European Society of Gene and Cell Therapy
Sociedad Española de Terapia Génica y Celular
Collaborative Congress 2013

25-28 October 2013
Palacio Municipal de Congresos de Madrid
Campo de las Naciones

www.esgct.eu • www.setgyc.es

VENUE INFORMATION

Palacio Municipal de Congresos de Madrid
Campo de las Naciones
Av. de la Capital de España Madrid, s/n
28042 Madrid

Phone: 00 34 91 722 04 00
Website: www.madridec.es

Underground station:
Line 8 (pink line), Station Campo de las Naciones

Bus (EMT Coaches):
- Línea 122, exit Intercambiador Avda de América
- Línea 112, exit Glorieta Mar de Cristal
- Línea 104, exit Cruz de los Caídos

By car:
M-40, exit number 7
A-II, exit Gran Vía de Hortaleza
M-11

Scan this QR code for location map

SPEAKER HOTEL INFORMATION:

Pullman Madrid Airport & Feria
Avd. Capital de España,10
Campo de las Naciones
28042 MADRID

Phone: +34 91/7210070
Email: H1606@accor.com

CONGRESS OFFICE INFORMATION

Gaëlle Jamar – Event Manager
Vanessa Sampson – Payment & Membership

PAYMENT QUERIES

Please go to the registration desk during the above opening hours

MEMBERSHIP QUERIES

Please go to the registration desk during the above opening hours

CONTACT NUMBER IN CASE OF EMERGENCY

Gaëlle Jamar, Event Manager
+44 7766 475379
office@esgct.eu

REGISTRATION & INFORMATION DESK

Friday 25 October 08.00-21.00
Saturday 26 October 08.00-20.00
Sunday 27 October 08.00-20.00
Monday 28 October 08.30-14.30

MADRID HOSPITALITY INFORMATION DESK

Located near exhibition booths on ground floor

THE HAGUE HOSPITALITY INFORMATION DESK

Located near Exhibiton Booths on ground floor

JOB OPPORTUNITIES AND OTHER IMPORTANT INFORMATION

Need a job? Need to hire? Want to share information with the other participants? Post resumes, employment opportunities or information on the designated boards located near the registration desk.

COATS AND LUGGAGE

For convenience, you can check in coats and luggage at level -1 in one of the green rooms of the auditorium. Please follow signs from reception.

SPEAKER READY ROOM

Monaco Room, level 1

ADDITIONAL MEETING ROOMS

The following meeting rooms on level 4 are available for hire, please enquire at information desk:
- Sala de Consejo (30 people)
- Sala Mirador (10 people)
- Despacho Alcalde (6 people)

Printed by Artes Graficas Gala S.L. Miguel Yuste, 36 – 28037 Madrid – 91 754 14 75
Design: www.catherinecharnock.co.uk

catherinecharnock CREATIVE

GOLD PARTNERS

AMGEN
BD
bluebirdbio
cellectis
cell pid
ciberer
EuroFancoLen
GENETHON
CURE THROUGH INNOVATION
MOLMED
PERSIST
TerCel

SILVER PARTNERS

AFMTELETHON
CURE THROUGH INNOVATION
andalusian initiative For advanced therapies
apceth
applied cell therapy
ATMI
GE Healthcare
BioReliance
Celgene
GENOSAFE
genzyme
A SANOFI COMPANY
MACS
Miltenyi Biotec
net CGD
bioMedica
PlasmidFactory
regenerar
uniQure
Universidad de Navarra
Hydro ZONES

CONGRESS SUPPORTERS

Asociación Española de ANEMIA DE FANCONI
AIPGENE
cima
CENTER FOR APPLIED MEDICAL RESEARCH
UNIVERSITY OF NAVARRA
BrainVectors
ciberNed
CELL CAM
enetrix
Mary Ann Liebert, Inc. publishers
NIMGenetics
New Integrated Medical Genetics
ReGenX
BIOSCIENCES
SANGAMO
Sangamo BioSciences, Inc.
SIGMA-ALDRICH
TiGENIX
Living Medicines
Universidad Carlos III de Madrid
VCN
BIOSCIENCES

CONTENTS

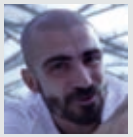
ESGCT EXCELLENCE AWARDS

We are delighted to present an exceptional field of award winners in 2013

Outstanding Achievement

Zelig Eshhar
The Marshall and Renette Ezralow Professor of Chemical and Cellular Immunology,
The Weizmann Institute of Science, Rehovot; Tel Aviv Sourasky Medical Center

Young Investigator



Luca Biasco
San Raffaele Telethon Institute for Gene Therapy, Milan

Jonathan Finn
Arthrogen BV, Amsterdam

Travel Grants

Kerstin Knoop, *University Hospital of Munich*
Xavier Bofill De Ros, *IDIBAPS/CIBERER*
Pietro Genovese, *San Raffaele Telethon Institute for Gene Therapy, Milan*
Ali Nowrouzi, *National Center for Tumor Diseases (NCT); German Cancer Research Center (DKFZ), Heidelberg*
Elisabeth Stein, *Technische Universität, Berlin*
Sofia Calado, *University of Algarve, Faro*
Simona Bramante, *Cancer Gene Therapy Group, Department of Pathology and Transplantation Laboratory, Haartman Institute, University of Helsinki*

Katharina Uhlig, *Paul-Ehrlich-Institut, Langen*

Lucie Pigeon, *Centre de Biophysique Moléculaire, CNRS UPR4301, Inserm and University of Orléans*
Jean-Baptiste Dupont, *UMR INSERM 1089, Nantes*

Javier Villadiego, *Instituto de Biomedicina de Sevilla-IBiS, HUVR/Universidad de Sevilla/CSIC*
Lester Suarez, *Centro de Investigación Médica Aplicada (CIMA), Division of Hepatology and Gene Therapy, University of Navarra, Pamplona*

Mathijs G.A. Broeren, *Radboudumc, Nijmegen, The Netherlands*

WELCOME ADDRESS

On behalf of the European and the Spanish Societies of Gene and Cell Therapy, we welcome you to the beautiful city of Madrid for the joint 2013 Congress. Europe has long been at the forefront of basic and translational research on gene transfer and clinical applications of gene and cell therapy. The ESGCT Annual Congress is the leading arena for reporting latest progress, nurturing scientific exchange among scientists and clinicians, training young researchers and disseminating knowledge to our stakeholders and society at large.

These are exciting times for gene and cell therapy. New generations of vectors have entered the clinical arena showing improved efficacy and safety. This has resulted in remarkable advances in the treatment of several diseases, such as retinopathies, haemophilia, immune-haematological and storage diseases as well as some types of cancer. Long-term safe and stable replacement of a previously missing or novel gene function is now within reach of clinical testing. The observed clinical benefits are increasingly supported by in-depth molecular follow up of human patients conducted with powerful new technologies. These advances are providing unprecedented insights into complex pathophysiological processes, such as stem cell activity, tumor progression and the deployment of an immune response.

Scientists have shown the potential of vector engineering to modify tissue tropism and have refined the genetic design of integrating vectors to increase safety and improve regulation of transgene expression. A novel generation of T-cell engineering strategies is showing the therapeutic potential of adoptive therapy in cancer patients. These advances are increasing our confidence and making our research goals even more ambitious. We now aim to achieve:

- stringently regulated expression of therapeutic transgenes,
- correction rather than replacement of malfunctioning genes,
- targeted delivery and lower toxicity of vector administration,
- reduced immune activation and induction of tolerance to the transgene product,
- improved engraftment of transplanted cells and tissue regeneration.

We work closely with regulatory authorities to improve the framework for the conduct of gene and cell therapy clinical trials. Most importantly, as gene and cell therapy are finally coming of age, we welcome an increasing involvement of the pharmacological industry in our field. Only through a close engagement with industry we can orchestrate regulatory adjustments and make our therapies a clinical reality.

The congress will report exciting new findings in all the major areas of cell and gene therapy: cancer, infectious or inherited disease, cell reprogramming, stem cell biology and regenerative medicine, vector targeting, gene editing strategies and nanotechnologies, immune response to vector and cell transplantation, imaging, manufacturing and ethical and regulatory issues of gene and cell therapy. Latest results from several clinical trials will be presented.

The scientific programme comprises six plenary sessions, 24 parallel sessions and two poster sessions, and will be preceded by a public engagement day on "Advanced Therapies in Rare Diseases" organised with the Spanish Institute for Rare Diseases (CIBERER). Additional satellite symposia include:

- an Early-Phase Clinical Trials Course;
- an Educational Program on Gene and Cell Therapy; and
- the 7th Annual "Stem Cell Clonality and Genome Stability Retreat" organised by the Transatlantic Gene Therapy Consortium.

In total the congress includes more than 100 lectures from invited speakers, over 70 competitively selected oral presentations as well as new "presented posters" sessions, which will give presenters of the top 80 posters the chance to present their work.

We hope that you will join the social events including the Welcome Reception, the posters parties and particularly the "Gala Dinner and Party" that will take place in the Casino de Madrid, officially declared as a building of National Cultural Interest. The gala dinner food will be a fusion of science and fine dining, prepared by Paco Roncero, the famous two Michelin star chef. After this amazing culinary experience those who wish to, can continue the fun at the Casino nightclub.

Madrid, Spain's capital, is a captivating city full of history, tradition, art, and bustling with life. Its many squares and parks invite you to relax after visiting one of its many top-class museums, or strolling around its romantic neighbourhoods. Madrid is a city that gets a hold on the visitor just after a few steps on its streets. Narrow, cobbled streets leading to busy squares and breath-taking historical architecture hidden around every corner maintain the city's quaint, Spanish vibe, yet its new metropolitan infrastructure and modern enhancements really represent Madrid's present and future.

Finally, we are indebted to patients' organisations, the EU, academic regional and corporate sponsors who supported us in the preparation, the management and also the costs of the Congress. Many of these organisations will be involved as exhibitors and we hope that you will take the time to visit them. We hope you will find the Congress as stimulating as we expect it to be, and thus look forward that you will enjoy both the outstanding scientific program and your stay in the charming city of Madrid.

Luigi Naldini
ESGCT President

Juan Bueren
SETGyC President
Organising Committee President

BOARDS

ESGCT Board

Luigi Naldini, Italy (President)
Nathalie Cartier-Lacave, France (Vice-President)
Christof von Kalle, Germany (Treasurer)
Len Seymour, UK (General Secretary)
Alessandro Aiuti, Italy
Robin Ali, UK
Hildegard Büning, Germany
Sarah Ferber, Israel
Hidde Haisma, The Netherlands
Zoltan Ivics, Germany

SETGyC Board

Juan Bueren, President
Javier García-Sancho, Vice-President
José Carlos Segovia, General Secretary
Josep Maria Canals, Treasurer
Isabel Fariñas, Vocal 1
Rubén Hernández, Vocal 2
Pablo Menéndez, Vocal 3
Juan Carlos Ramírez, Vocal 4
Pilar Martín-Duque, Vocal 5

Local Organising Committee

Juan Bueren, President
José Carlos Segovia
Guillermo Güenechea
Ramón Alemany
Antonio Bernad
Fátima Bosh
Marcela del Río
Damián García-Olmo
Gloria González Aseguinolaza
José López-Barneo
Felipe Prosper
Ángel Raya

Organisers:

Wats.on Consultancy: Renée Watson, Gaëlle Jamar, Vanessa Sampson, Mel Cunningham

With special thanks to

SCIENTIFIC COMMITTEES

Cardiovascular diseases
Andy Baker (Chair)
Seppo Ylä-Herttuala (Chair)
Mauro Giacca
Moshe Flugelman
Keith Channon
Patrick Most

Neurological and muscular diseases
Nicole Déglon (Chair)
Maurilio Sampaolesi (Chair)
Nathalie Cartier (Chair)
Nicholas Mazarakis
Dominic Wells
Anders Björklund

Viral vectors
Hildegard Büning (Chair)
Luigi Naldini (Chair)
Rob Hoeben
Akseli Hemminki
Anna Salvetti
Alberto Epstein
Els Verhoeyen

Genetic and metabolic diseases
Edvard Smith (Chair)
Thierry VandenDriessche (Chair)
Fatima Bosch
Manuel Grez
Robin Ali
Beat Thony

Non-viral vectors
Daniel Scherman (Chair)
Hidde Haisma (Chair)
Ernst Wagner
J.P. Behr
George Dickson
Zoltan Ivics

Infection, immune and vaccines
David Klatzmann (Chair)
Mary Collins (Chair)
Naomi Taylor
Ben Berkhout
Dorothee Von Laer
Zelig Eshhar

Stem cells and reprogramming
Stefan Karlsson (Chair)
Christopher Baum (Chair)
Juan Bueren
Tim O'Brien
Catherine Verfaillie
Katarina Le Blanc
Marina Radrizzani
Willem Fibbe

Ethics and regulatory affairs
Odile Cohen-Haguenauer (Chair)
Klaus Cichutek (Chair)
Richard Ashcroft
Alastair Kent
Gösta Gahrton
Serge Braun
Otto Merten
Martin Schleef
Klaus Kühlcke
Michael Fuchs

PROGRAMME AT A GLANCE

FRIDAY 25 OCTOBER 2013

EARLY PHASE CLINICAL TRIALS TRAINING COURSE *Pullman Hotel, Magna Room* *Sponsors: Cellpid*		P. 44
09.00	Introduction	
	Planning an academic trial – the sponsor's perspective	
	Production of vector and genetically modified stem cells	
	The role of QP in assessing ATMPs	
	AAV gene therapy for haemophilia	
	Lentiviral vector GT for beta-thalassemia	
	Gammaretro and lentiviral vectors for the gene therapy of X-linked Chronic Granulomatous Disease	
11.30	Break	
12.00	EU regulations for ATMP and clinical trials	
	Ensuring GCP compliance, patient safety and data integrity	
	Glybera® approval: a road map for advanced therapies in the orphan space	
	Phase Ib/IIa, escalating dose, single blind, clinical trial to assess the safety of the intravenous administration of expanded allogeneic adipose-derived mesenchymal stem cells (eASCs) to refractory rheumatoid arthritis (RA) Patients	
	Regulatory challenges in development of lentiviral *ex vivo* gene therapy products	
	ATMP in the EU; the long and winding road	
14.00	Lunch – exhibition and congress (at the Palacio Municipal de Congressos)	

FRIDAY 25 OCTOBER 2013

PUBLIC SESSION: ADVANCES ON THE GENE THERAPY OF RARE DISEASES ***Sala Madrid***		P. 46
09.15	Inicio de acreditación y bienvenida	
09.45	Bienvenida	
10.00	Papel de la terapia génica en enfermedades raras	
10.30	Terapia génica en anemia de Fanconi	
10.50	Terapia celular y génica epidermólisis bulosa	
11.10	Terapia génica de porfirias hepáticas	
11.40	Pausa Cafe	
12.10	Terapia génica de mucopolisacaridosis	
12.30	Gene therapy of adrenoleukodistrophy	
13.00	Gene therapy of immunodeficiences	
13.30	Terapias avanzadas, la visión de los afectados	
13.45	Conclusiones	

EDUCATION DAY			P. 48
10.00	**1: Cancer gene therapy** *Sala Paris*	**2: Stem cells and tissue engineering** *Sala Berlin* *Sponsor: CellCAM*	
	Bases of gene therapy in leukemias	Neural stem cells	
	Recent developments in gene therapy of solid tumours	Hepatic progenitor cells in liver regeneration	
11.30	Morning break		
12.00	**3: Gene therapy in rare diseases** *Sala Paris* *Sponsor: Genzyme*	**4: From bench to bedside** *Sala Berlin* *Sponsor: CellCAM*	
	Translational research in the *ex vivo* gene therapy of monogenic diseases	Clinical trials with mesenchymal stem cells in wound healing	
	Progress and challenges of *in vivo* gene transfer with AAV vectors	Ethic issues in cell and gene therapy	
13.30	Lunch – Exhibition and poster halls		

PROGRAMME AT A GLANCE

FRIDAY 25 OCTOBER 2012

MAIN CONFERENCE					
15.00	**Inaugural session – Auditorium** *Sponsor: Molmed*				
15.45	**1: Cell reprogramming** *Auditorium* *Sponsor: Molmed*				P. 50
17.15	Afternoon break				
17.45	**1a: Transposon and non viral gene transfer** *Auditorium* *Sponsor: GE Healthcare*	**1b: Biology and manipulation of stem cells** *Sala Madrid* *Sponsor: NimGenetics*	**1c: Immune responses in gene and cell therapy** *Sala Paris* *Sponsor: Genosafe*	**1d: Imaging in cell and gene therapy** *Sala Berlin*	P. 50
19.30	**Welcome reception**				

SATURDAY 26 OCTOBER 2013

MAIN CONFERENCE					
09.00	**2. Stem cells and stem cell niche** *Auditorium* *Sponsor: Amgen*				P. 56
10.30	Morning break				
11.00	**2a: Oncolytic viral therapy** *Auditorium* *Sponsor: ATMI, JSGT*	**2b: Primary immuno-deficiencies** *Sala Madrid* *Sponsor: Cellpid*	**2c: Muscular and bone diseases** *Sala Paris* *Sponsor: Généthon*	**2d: Bioprocessing of cell and gene therapy products** *Sala Berlin* *Sponsor: BioReliance*	P. 56
12.45	ESGCT General Assembly – Auditorium				
12.45	Lunch – Exhibition and poster halls				
14.15	**3: Gene and cell therapy in regenerative medicine** *Auditorium* *Sponsor: TerCel*				P. 60
15.45	Afternoon break				
16.15	**3a: Cell reprogramming** *Auditorium* *Sponsor: AIAT*	**3b: Inflamatory and autoimmune diseases** *Sala Berlin* *Sponsor: regenerar*	**3c: Aging and genetic instability syndromes** *Sala Paris* *Sponsor: Ciberer, EuroFancolen*	**3d: Immunotherapy of cancer** *Sala Madrid* *Sponsor: Cellectis*	P. 62
18.00 18.30	Poster session A (Odd numbers) – Poster halls Presented posters session A – see table on page 68 for details				
21.00	Speakers' Dinner – by invitation only *Coaches will depart from Pullman Hotel at 20.15*				

PROGRAMME AT A GLANCE

SUNDAY 27 OCTOBER 2013

MAIN CONFERENCE					
08.45	**4: Hematopoietic stem cell gene therapy** *Auditorium* *Sponsor: Généthon, Persist*				P. 72
10.45	Morning break				
11.15	**4a: Red blood cell diseases** *Sala Madrid* *Sponsor: bluebirdbio*	**4b: Cardiovascular diseases** *Sala Paris* *Sponsor: Miltenyi Biotec*	**4c: Neural diseases** *Auditorium* *Sponsor: CiberNed*	**4d: Nanotechnology and RNA therapeutics** *Sala Berlin*	P. 72
13.00	SETGyC General Assembly – Auditorium				
13.00	Lunch – Exhibiton and poster halls				
14.30	**5: Gene and cell therapy for cancer** *Auditorium* *Sponsor: Apceth*				P. 78
16.00	Afternoon break				
16.30	**5a: Blood coagulation diseases** *Auditorium*	**5b: Regenerative medicine** *Sala Paris* *Sponsor: Universidad de Navarra*	**5c: Gene editing** *Sala Madrid* *Sponsor: Sigma-Aldrich, Sangamo*	**5d: Skin diseases** *Sala Berlin* *Sponsor: Universidad Carlos III de Madrid, JSGT*	P. 78
18.15 18.30	Poster session B (Even numbers) – Poster halls Presented posters session B – see page 84 for details				
21.00	Gala dinner – Casino de Madrid, Alcalá 15, 28014 Madrid (Metro Sevilla) *Coaches will depart from front of Palacio Municipal at 20.15*				

MONDAY 29 OCTOBER

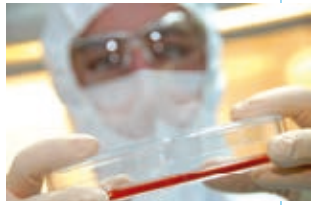
MAIN CONFERENCE					
09.15	**6a: Metabolic and lysosomal storage diseases** *Sala Madrid* *Sponsor: Aipgene*	**6b: Ocular diseases** *Sala Berlin* Sponsor: ReGenX Biosciences	**6c: Immuno-therapy of cancer (II) and infectious diseases** *Auditorium* *Sponsor: Cellectis*	**6d: Viral vector developments** *Sala Paris*	P. 88
11.00	Morning break				
11.30-13.30	**Presidential symposium awards ceremony** *Auditorium* *Sponsor: BD*				P. 92

GenoSafe

Safety and Efficacy of biotherapeutic products

From research to clinical trials, we meet our client's specific needs by performing custom studies in strict compliance with regulatory requirements

- **Development and validation of specific assays and analytical methods**
- **Custom samples analysis**



Molecular assays

- Biodistribution and shedding studies
- Custom qPCR and RT-qPCR assays

Viral vectors

- Titration of viral particles and infectious genomes
- Detection of recombinant particles
- Detection of cell or plasmid-specific sequences

Immunology

Characterization of:
- Inflammatory responses
- Humoral responses
- Cell-mediated responses

Cell lines

- Customized cell line engineering
- Stem cell characterization

Clinical trials

- Patient Follow-up

Address
GenoSafe
1 rue de l'internationale
BP 40064
91002 Evry Cedex
France

www.genosafe.com

Shareholders
Genethon (60%)
AFM (40%)
Founded 2003

Contact
Vincent Zuliani
Phone: +33(0)1.69.47.11.57
Fax: +33(0)1.69.47.11.61
contact@genosafe.com

PARTNERS

GOLD PARTNERS

Amgen discovers, develops, manufactures, and delivers innovative human therapeutics. A biotechnology pioneer since 1980, Amgen was one of the first companies to realise the potential in the new science to bring safe, effective medicines from lab to manufacturing plant to patient. Amgen therapeutics has changed the practice of medicine, helping millions of people around the world in the fight against cancer, kidney disease, rheumatoid arthritis, bone disease, and other serious illnesses. With a deep and broad pipeline of new medicines, Amgen remains committed to advancing science to dramatically improve people's lives.

www.amgen.com

BD Biosciences is a world leader in bringing innovative diagnostic and research tools to life science researchers, clinical researchers, laboratory professionals and clinicians who are involved in basic research, drug discovery and development, biopharmaceutical production and disease management. The BD Biosciences segment is focused on continually advancing the science and applications associated with cellular analysis and products that help grow living cells and tissue. Products/Services:1) Fluorescence-activated cell sorters and analysers; 2) Monoclonal antibodies and kits for cell analysis; 3) Reagent systems for life science research; 4) Cell culture media and supplements for biopharmaceutical manufacturing.

www.bdbiosciences.com

Bluebird bio is developing innovative gene therapies for severe genetic disorders. The company's proprietary platform treats genetic diseases by placing a functional gene into the patient's extracted bone marrow stem cells, and transplanting these corrected stem cells back into the patient. Bluebird bio has two later stage clinical products in development for childhood cerebral adrenoleukodystrophy and beta-thalassemia/sickle cell anemia.

www.bluebirdbio.com

The Cellectis Group is based on a highly specific DNA engineering technology. Its application sectors are within human health, agriculture and bio-energies. Co-created by André Choulika, Chief Executive Officer, Cellectis Group is one of the world leading companies in the field of genome engineering. The Group has a workforce of 230 employees working on five sites worldwide. AFM, Dupont, BASF, Bayer, Total, Limagrain and Novo Nordisk are some of the Group's clients and partners.

www.cellectis.com

GOLD PARTNERS

The FP7-CELL-PID European project utilises genetically modified HSC and their descendants as immunotherapeutic cells to build a healthy immune system in primary immune-deficiency patients. It gathers together clinical pioneers, scientists and industrial partners in the advanced therapies field and aims to develop broad clinical application of safe cell-based therapies.

http://cordis.europa.eu

Rare diseases, affecting around three million Spanish people, are a huge social and health problem. The Centre for Biomedical Network Research on Rare Diseases (CIBERER), is a network structure set up at the initiative of the Instituto de Salud Carlos III, which furthers the excellence research done in this country, with the aim of finding diagnoses and therapies for those affected as quickly as possible.

www.ciberer.es

EuroFancoLen is a collaborative project with an innovative approach to developing, for the first time, an efficient and safe gene therapy of FA based on two recent innovations:
1) Discovery of potent HSC mobilisers, such as plerixafor
2) Development of a new lentiviral vector by members of this Consortium, designed as Orphan Drug by the Europan Commission in December 2010

The main objective of this project is, therefore, the development of a multicentric Phase I/II gene therapy trial for FA-A patients, based on the genetic correction of plerixafor+G-CSF mobilised HSCs with the novel lentiviral vector, accompanied by comprehensive and groundbreaking safety and efficacy patient monitoring studies.

www.eurofancolen.eu

Généthon develops and manufactures gene therapy products for rare diseases with the goal of making these innovative treatments available to patients. To meet this challenge, Généthon has assembled the technical and human resources needed to accelerate the medical application of scientific discoveries arising from fundamental research. Strong translational research programmes (at preclinical and clinical stages) engage multi-disciplinary teams and are supported by a first-rate technological platform and cGMP capacity. Généthon currently sponsors several early-phase gene therapy clinical trials (including an international trial).

www.Généthon.fr

GOLD PARTNERS

MolMed is a medical biotechnology company focused on research, development and clinical validation of innovative therapies. In cell and gene therapy, our GMP Division offers high level expertise to develop, conduct and validate custom studies, optimise and scale-up manufacturing projects, devising innovative testing procedures and addressing the unique test specifications required for novel genetic therapies through the development and manufacturing of viral vectors and the transduction of patients hematopoietic stem cells. (Aiuti et Al., Science. 2013 Aug 23;341(6148):1233151 - Biffi et Al., Science. 2013 Aug 23;341(6148):1233158 - and Aiuti et Al., N Engl J Med. 2009 Jan 29;360(5):447-58)

www.molmed.com

PERSIST project explores the use of highly innovative gene-modifying and delivery technologies. It capitalises on recent discoveries in gene expression control to develop radical solutions to precise control the fate and expression of exogenous genetic information in gene therapy. The project combines more than 20 of Europe's outstanding experts from eight countries in the field of genetic engineering for gene expression.

www.persist-project.eu

The Spanish cell therapy network (TerCel) is a collaborative research project organised by the Spanish National Institute of Health Carlos III that started in 2003, to promote research in cell therapy and to translate the scientific advances in this field into clinic medicine. Based on a multidisciplinary approach for the interaction and cooperation between 33 groups of basic and clinical scientists across Spain, the main objective of TerCel is to develop new medical therapies based on the use of stem cells for cardiovascular diseases, neurodegenerative diseases and osteo-articular, immune-hematologic and metabolic diseases.

www.red-tercel.com

SILVER PARTNERS

The Andalusian Initiative for Advanced Therapies (IATA) is a publicly funded organisation created by the regional government of Andalusia (Junta de Andalucía) to support activities of R&D&i in the fields of cell therapy and regenerative medicine, clinical genetics, genomic medicine and nanomedicine. We act as an interface between academia (hospitals, universities and other research institutions) and the industry (biotechnological and pharmaceutical companies) to facilitate research and technology transference. Being part of such a multidisciplinary system makes the IATA an ideal partner offering comprehensive support for translational research, regulatory expertise and advisory support and a unique European master degree in manufacturing of advanced therapies medicinal products. We are looking to establish collaborations based not only on offering services but specifically in creating partnerships within a risk- benefit sharing model to enhance and accelerate the development of new products.

www.juntadeandalucia.es/terapiasavanzadas/en/central-office

AFM (French Muscular Dystrophy Association) has a single objective: to defeat neuromuscular diseases, which are devastating muscle-wasting diseases. Created in 1958 by a group of patients and their families, and recognised as being of public utility in 1976, it has set itself two missions: curing neuromuscular diseases and reducing the disabilities they cause.

www.afm-france.org

Apceth GmbH & Co. KG is a clinical stage biopharmaceutical company focused on the development of native and genetically modified cell therapeutics ("Advanced Therapy Medicinal Products", ATMPs). We combine the principles of adult stem cell biology with pioneering technologies and the highest standards for GMP manufacturing and quality management, according to European and international guidelines.

We offer contract GMP manufacturing and related services using our expertise, high standards and state-of-the-art GMP/BSL2 facilities of more than 600 m2 of cleanroom area (class ISO 8, ISO 7, ISO 5), quality control units and R&D laboratories. Our manufacturing license covers native and genetically modified cell therapeutics, according to §13 and §20b of the German Medicines Act (AMG).

We are the first company worldwide to receive approval for a phase I/II clinical trial with genetically modified mesenchymal stem cells for the treatment of advanced gastrointestinal cancer (start 10/2013). In parallel, our first clinical phase I/II trial for the treatment of critical limb ischemia with our somatic cell therapeutic is currently ongoing.

www.apceth.com

SILVER PARTNERS

ATMI is a global leader in enabling process materials and process technology for semiconductor, display and life science industries. At ATMI, process ingenuity unleashes new process possibilities for customers.

www.atmi-lifesciences.com

BioReliance was acquired by Sigma-Aldrich Corporation, in January, 2012 to supply customers access to a powerful, single-point provider whose products and services span the drug discovery, development and commercialisation pipeline. As part of the SAFC business unit, BioReliance is a leading provider of cost-effective contract services, offering more than 1,000 tests or services related to biologics safety testing, specialised toxicology and animal health services. BioReliance provides outsourced services to thousands of customers from most healthcare disciplines and is the largest provider of safety testing services focused on the rapidly growing biologics sector of the pharmaceutical and biotechnology industries. BioReliance has over 700 employees and has laboratory operations in Rockville, MD and Scotland.

www.bioreliance.com

Celgene is a global biopharmaceutical company committed to improving the lives of patients with certain types of cancer and other rare incurable diseases. Our mission is to deliver truly innovative and life-changing drugs for our patients. As a company we work to build a major global biopharmaceutical corporation while focusing on the discovery, the development, and the commercialisation of products for the treatment of cancer and other severe, immune, inflammatory conditions. There are more than 300 clinical trials at major medical centers using compounds from Celgene. Investigational compounds are being studied for patients with incurable hematological and solid tumor cancers, including multiple myeloma, myelodysplastic syndromes, chronic lymphocyte leukemia (CLL), non-Hodgkin's lymphoma (NHL), myelofibrosis, small cell lung cancer and prostate cancer.

www.celgene.es

GE Healthcare Life Sciences provides tools for drug discovery, biopharmaceutical manufacturing and cellular technologies, so research scientists and specialists around the world can be more productive, effective and creative. Our vision is to be the start-to-finish bioprocessing solution provider, the partner of choice in cell and protein research, and the leader in life sciences services. Building on our broad expertise across life sciences, we are firmly committed to help researchers around the world discover new ways to predict, diagnose and treat disease.

www.gelifesciences.com

SILVER PARTNERS

GenoSafe is a CSO specialised in the evaluation of the quality, efficacy and safety of gene and cell therapy products. We support through research stages to final clinical phases: from study design, development/validation of analytical methods, and product testing to control of viral vectors batches (rAAV, rHIV, rMLV), preclinical evaluation, clinical trial and finally, patient follow-up.

www.genosafe.org

Genzyme has pioneered the development and delivery of transformative therapies for over 30 years. Founded in 1981 in Boston, Massachusetts, Genzyme evolved from a tiny start-up with just a handful of employees to one of the world's leading biotech companies. Acquired by Sanofi in 2011, Genzyme now benefits from the reach and resources of one of the world's largest pharmaceutical companies, with a shared commitment to improving the lives of patients.

Genzyme has long been known for our expertise in the class of rare genetic diseases known as lysosomal storage disorders (LSDs). LSDs remain the heart of our company today, but we have also expanded – through both in-house development and strategic acquisitions and partnerships – to other disease areas such as thyroid cancer and multiple sclerosis.

www.genzyme.com

Miltenyi Biotec is a global provider of products and services that advance biomedical research and cellular therapy. Since 1989, we have developed innovative and reliable technologies for scientists and clinicians around the world. Our integrated portfolio of tools covers techniques of sample preparation, cell separation, flow cytometry, cell culture, molecular analysis, and preclinical imaging. The MACS® brand has set standards worldwide and is trusted across basic, translational, and clinical research settings. Our expertise covers research areas like immunology, stem cell biology, neuroscience, and cancer, and clinical research areas that include hematology, graft engineering, as well as apheresis. We are committed to supporting scientists and the scientific community – from dedicated technical support to comprehensive training courses at our MACS Academy. Today, we are more than 1,300 employees in 22 countries – all dedicated to providing solutions that empower scientific discovery and advance cellular therapy.

www.miltenyibiotec.com

SILVER PARTNERS

Net4CGD, Gene Therapy for X-linked Chronic Granulomatous Disease (CGD), is a large-scale integrating project in the health research of the European commission 7th Framework Programme (FP7). The Net4CGD project is focused on the clinical development of a new orphan drug of gene therapy for X-linked form of CGD to become a new treatment option for patients. The Net4CGD consortium consists of 11 partners institutions and is coordinated by Genethon, France.

Oxford BioMedica (LSE: OXB) is a biopharmaceutical company specialising in the development and commercialisation of innovative gene-based medicines. The Company has a platform of gene delivery technologies, which are predominately based on highly engineered viral systems.

www.oxfordbiomedica.co.uk

PlasmidFactory is Europe's leading contract manufacturer for plasmid DNA. Production ranges from research to industrial scale. We produce plasmids in modern laboratories to the highest quality of standards and according to your individual wishes.

www.PlasmidFactory.com

REGENER-AR stands for Bringing Regenerative Medicine into the market: Allogeneic eASCs Phase IB/IIA clinical trial for treating Rheumatoid Arthritis. REGENER-AR is a clinical translational collaborative project that is developing treatments for Rheumatoid Arthritis (RA) by exploiting the biology of living human expanded allogeneic mesenchymal adult stem cells extracted from adipose tissue (eASCs). To accomplish this goal, we test an advanced cellular therapy through a phase Ib/IIb clinical trial in order to define the safety and feasibility of the systemic administration of allogeneic eASCs in patients with RA. We will also seek to overcome difficulties and barriers that prevent cell therapy becoming a widely available product by addressing issues related to production scale-up, effective clinical treatment and regulatory affairs compliance.

www.regenerar.eu

SILVER PARTNERS

uniQure is the world leader in the development of human gene based therapies. uniQure's Glybera, a gene therapy for the treatment of lipoprotein lipase deficiency has been approved in the European Union, and is the first approved gene therapy in the Western world. uniQure's product pipeline of gene therapy products in development comprise of hemophilia B, acute intermittent porphyria, Parkinson's disease and SanfilippoB. Using adeno-associated viral (AAV) derived vectors as the delivery vehicle of choice for therapeutic genes, the company has been able to design the world's first stable and scalable AAV manufacturing platform, validated in the approval process of Glybera. This proprietary platform can be applied to a large number of rare (orphan) diseases caused by one faulty gene.

http://uniqure.com

The Cell Therapy Area of the University of Navarra (UNAV) was established in 2002 and is headed by Dr. Felipe Prósper. Research in his laboratory is focused on the role of stem cells for treatment of cardiovascular diseases and involves from basic studies to preclinical animal models to several clinical trials with ATMP. The Department includes a certified GMP laboratory. The Clínica Universidad is the academic medical centre of the University of Navarra and functions closely with the Faculty of Medicine, Sciences, Pharmacy, the Nurse School and the Centre for Applied Medical Research.

http://www.unav.edu

CONGRESS SUPPORTERS

The initial diagnosis of Fanconi anemia causes shock, confusion, denial, anger and a sense of impotence in the affected family. This reaction is perfectly understandable and natural; for this reason, we created The Spanish Association of Fanconi anemia, to offer all the information and support we can. In Spain, there is a large team of physicians and scientists collaborating to combat this disease. In November 2002, the Spanish Group for the study and treatment of Fanconi anemia published its first newsletter, Patient and Family Specialists supported by CIEMAT, the Fundación Marcelino Botin and the Autonomous University of Barcelona. It is our observation that after diagnosis, you may be looking for more in depth information about the disease, which we provide on our website. You will also find links to different organisations, including Fanconi anemia José Carreras Foundation for the fight against leukemia, Marcelino Botin Foundation and the CIEMAT.

www.asoc-anemiafanconi.es

The Project AIPGENE is a collaborative project, involving seven partners under the European Commission's 7th framework programme. AIPGENE is investigating new approaches for the treatment of Acute Intermittent Porphyria (AIP) disease. The project is co-ordinated by the Center for Applied Medical Research (CIMA), a European center of excellence for research, Pamplona, Spain. The other partners implicated are uniQure (Netherlands), Clínica Universidad de Navarra (Spain), the Karolinska University Hospital (Sweden), the National Center for Tumor Diseases (Germany), Digna Biotech (Spain) and the Hospital 12 de Octubre (Spain).

The principal aim of the AIPGENE project is to develop the potential of the orphan drug AAV5-AAT-PBGD for the treatment of the rare disease AIP. Successful completion of this project will represent an important advance in the clinical and therapeutic management of AIP and of metabolic liver diseases in general, as well as in our understanding of their physiopathology.

www.aipgene.org
www.cima.es
http://europa.eu

CONGRESS SUPPORTERS

BrainVectors (From Brain Gene Transfer Towards Gene Therapy: Pharmacological Assessment of AAV, CAV-2 and LV) is a EC-funded Industry- Academia Partnership and Pathways (IAPP) network of 10 academic and industrial laboratories having cutting edge expertise on viral vectors, brain gene transfer and immunology. The research program focuses on the development of improved TetOn expression cassettes and their evaluation when delivered by AAV, CAV-2 and LV into 2D/3D primary neuronal cells and *in vivo* in animal models of Parkinson's disease. We do a comparative analysis of the transduction efficiency, bio-distribution, sensitivity to the inducer as well as the cellular and humoral immune responses to the regulatable system and to the vectors. The work is carried out through an intense program of cross-sector secondments (academia and industry) involving the staff members of the consortium institutions and three recruited researchers. The BrainVectors project is coordinated by Drs Liliane Tenenbaum (CHUV, Lausanne, CH) and Mauro Mezzina (EASCO, Paris, F)

www.brainvectors.org

The Biomedical Research Networking center in Bioengineering, Biomaterials and Nanomedicine (CIBER-BBN) is one of nine CIBER consortia, created under the leadership of the Carlos III Health Institute (ISCIII) to promote research excellence and build a critical mass of researchers in the field of Biomedicine and Health Sciences.

The research programs of the CIBER-BBN are: Bioengineering and biomedical imaging, Biomaterials and tissue engineering and Nanomedicine, with the Centre's research aimed at developing systems for prevention, diagnosis and monitoring and related technologies for specific treatments such as Regenerative Medicine and Nanotherapies.

CIBER-BBN aims to increase the research capabilities of the member groups by sharing and better utilizing resources and coordination and promotion of research efforts. The CIBER will increase the Spanish presence in international decision-making forums and research networks. Together we aim to foster high-level and cutting edge training, technology and translation in Spain.

www.ciber-bbn.es

CONGRESS SUPPORTERS

CIBERNED is the acronym for "Centro de Investigación Biomédica En Red Enfermedades Neurodegenerativas", a consortium that includes about 60 of the most competitive Spanish groups working on the molecular bases of neurodegenerative diseases. Group leaders are from universities, Spanish Research Council (CSIC) or hospitals from all over Spain, although most of the CIBERNED members are based in Madrid or Barcelona. Research done by CIBERNED groups has a strong "translational" orientation. Therefore, besides research on pathogenesis and molecular bases of diseases (particularly Alzheimer's and Parkinson's diseases) many of these groups have also a strong focus on the development of new cell and gene therapies. CIBERNED is mainly supported by the Spanish Ministries of Health and Research through the Instituto de Salud Carlos III (Spanish Medical Research Council).

www.ciberned.es

CellCAM "A New generation of safer and more efficient cellular treatments" is a consortium of the Biomedicine R+D Program 2010 of the Autonomous Community of Madrid. The consortium is composed of 8 groups from different research centres of Madrid with international prestige, coordinated by Dr. Juan A. Bueren (CIEMAT) and including Dr. A. Bernad (CNIC), Dr. J.G Castro (BIOC-ISCIII), Dr. F. Fernández-Avilés (HGUGM), Dr. M. Del Río (Uc3M), Dr. D. García Olmo (idiPAZ), Dr. A. Zapata (UCM), Dr. M. Ramírez (FIBHUJNS). There are two associated companies TIGENIX S.A.U. in research activities and Janus Developments S.L. that carries out research management activities. The main objective of this Consortium is to consolidate the translational research in cellular therapy to develop treatments for different diseases with a potential curative treatment by these strategies.

http://projects.ciemat.es/web/cellcam/

Genetrix, comprises of a group of biotech companies that develop and commercialise innovative products in the following markets:

- Tools to amplify, repair and sequence DNA, which are essential for the main molecular biology techniques used in the lab and playa key role in the up-and-coming personalised medicine field.
- Cell therapy, based on adult stem cells, which has proved to have enormous potential to tackle diseases that currently do not have established therapeutic treatment, such as myocardial infarction, rheumatoid arthritis and Parkinson's disease.

The Genetrix Group structure has been designed to transform academic results into:

- Innovative sanitary products and services
- Molecular Biology tools with global market potential.

Our focus is on high value investments, with the support of relevant industrial and financial partners for every project.

www.genetrix.es

CONGRESS SUPPORTERS

Human Gene Therapy is the premier journal covering all aspects of human gene therapy, including DNA, RNA, and cell therapies. HGT has now expanded into two parts to include *HGT Methods*, a bimonthly journal focused exclusively on protocols, new tools, lab techniques, and procedures. The unique package of *Human Gene Therapy* and *HGT Methods* provides 18 issues of essential research, technologies, translation, and applications to promote the development of gene therapy products into effective therapeutics for treating human disease. The journal publishes original investigations into the transfer and expression of genes and improvements in vector development, delivery systems, and animal models, including cancer, AIDS, heart disease, genetic disease, and neurological disease.

www.liebertpub.com/hum

NIMGenetics is dedicated to the design, innovation, development and commercialisation of the latest generation in biomedical technology products and services for genetic diagnostics.

One of our strengths is in genomic data analysis and interpretation done by the NIMGenetics team. The NIMGenetics team uses two of the most powerful and state-of-the-art technologies: array CGH, with our own designs manufactured by Agilent Technologies and next generation sequencing using ion torrent. These technologies are a hundred times more potent and accurate than traditional karyotype. We use them for: Prenatal and Postnatal Diagnostics, Oncology, Cardiology and Stem Cells Quality Control. NIMGenetics also provides support services for R&D institutions.

NIMGenetics was created by Dr. Cigudosa (CNIO), Dra. Álvarez (CNIO) and Dr. Samper (CNIC). This team is a national reference in molecular and diagnostic cytogenetics, as well as in stem cells biosafety. NIMGenetics has 14 employees, 7 of them with a PhD in biomedicine. NIMGenetics is an authorised center of analytic diagnostics with a genetic unit, and has the accreditation as an Agilent Certified Service Provider (CSP) in Spain.

www.nimgenetics.com

ReGenX BioSciences is leading the effort to translate promising gene delivery applications into a pipeline of next generation personalised therapies for a range of severe diseases with serious unmet needs. Our proprietary NAV™ technology is based on NAV vectors, the next generation of recombinant adeno-associated virus (rAAV) vectors, including NAV rAAV8, NAV rAAV9, and NAV rAAVrh.10. We believe that the NAV technology represents the potential promise of curing the root cause of disease rather than the symptoms, and we are committed to establishing best in class standards for our NAV vectors.

www.regenxbio.com

CONGRESS SUPPORTERS

Sangamo BioSciences, Inc. is developing novel zinc finger DNA-binding proteins (ZFPs), for therapeutic gene regulation and genome editing. Sangamo has ongoing Phase 2 clinical trials to evaluate safety and efficacy of a ZFP Therapeutic® for the treatment of HIV/AIDS. Other therapeutic programs are focused on monogenic diseases. Sangamo engineers sequence-specific ZFP Nucleases (ZFNs) for gene modification and ZFP transcription factors (ZFP TFs) for gene regulation. The company has strategic partnerships with Shire, Dow AgroSciences and Sigma-Aldrich Corporation.

www.sangamo.com

A reliable, single-source research partner providing reagents, technologies and industry-leading service to the life science and high technology markets. Our strengths lie in scientific knowledge, innovation, high-quality products and reliable delivery to support consistent, predictable results and save researchers time. The Company operates in 35 countries with customers in 160 countries. Our objective is to provide excellent service worldwide.

We provide a range of global solutions for research and manufacturing customers across our brands: Sigma® Life Science; Aldrich® Chemistry; Fluka® Analytical; Supelco® Analytical; SAFC®.

www.sigma-aldrich.com

TiGenix is a leading European company with a strong adult cell therapy program in their clinical stage pipeline and into their commercial products. The company's lead product, ChondroCelect®, for cartilage repair in the knee, is the only approved cell-based product in Europe, and is currently being launched across Europe. TiGenix's adipose derived allogeneic stem cell platform has been extensively validated. TiGenix is based out of Leuven (Belgium) and has operations in Madrid (Spain), and Sittard-Geleen (the Netherlands).

www.tigenix.com

CONGRESS SUPPORTERS

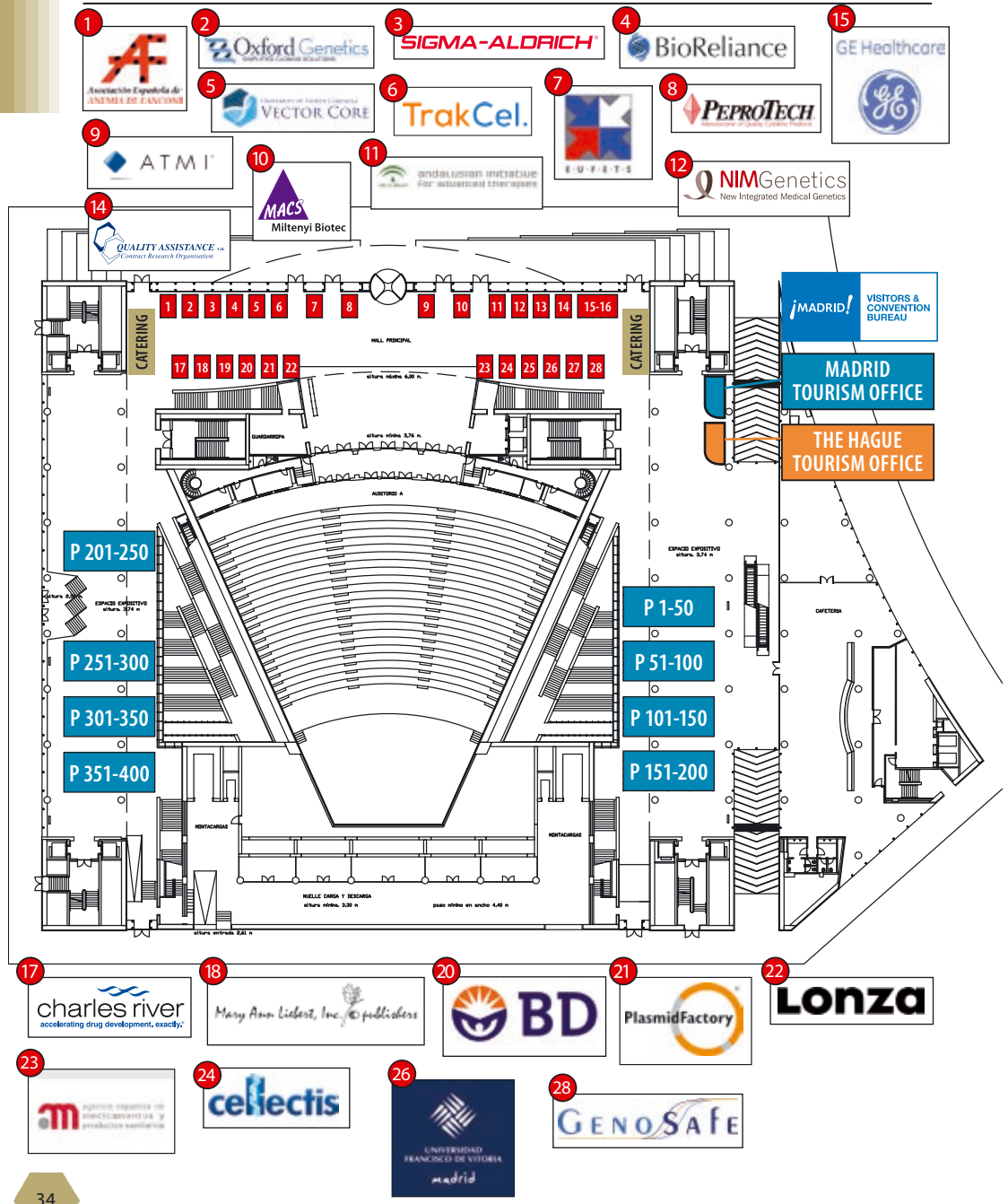
Universidad Carlos III de Madrid: Our group has strong knowledge in pre-clinical assessment of skin regeneration, skin disease modelling and gene therapy combined with extensive experience in clinical cell therapy approaches for skin diseases. Our group established the first platform for Epidermolysis Bullosa diagnostics in Spain and, promoted and coordinated other task forces leading to multidisciplinary patient management.

www.uc3m.es

VCN Biosciences is a privately-owned company focused in the development of new therapeutic approaches for tumors that lack effective treatment. The company uses oncolytic adenovirus technology platform to design highly selective and efficient agents that replicate and self-amplify exclusively in tumor cells. The selectivity of VCN oncolytic adenoviruses allows their systemic administration, which is especially relevant for the treatment of disseminated cancer. Contrary to chemotherapy, the ability of oncolytic virus to self-amplify in tumor cells results in an effective dose increase with time. These properties highlight VCN candidates as promising alternatives for the treatment of refractory tumors such as pancreatic adenocarcinomas, which is the current tumor target for its most advanced candidate, VCN-01.

www.vcnbiosciences.com

EXHIBITION HALL



POSTERS

POSTERS BY CATEGORY	
Odd numbers will be presented on Saturday *Even numbers will be presented on Sunday*	
P001 – P042	Non viral vectors, nanotechnology and RNA therapies
P043 – P078	Viral vector developments
P079 – P104	Gene and cell targeting
P105 – p122	Stem cells biology
P123 – P154	Bioprocessing, manufacturing and regulatory issues
P155 – P169	Cell reprogramming
P170 – P181	Lympho-hematopoietic gene therapy
P182 – P196	Cardiovascular diseases
P197 – P227	Neuro-muscular diseases
P228 – P244	Bone, other diseases and ageing
P245 – P259	Skin diseases
P260 – P280	Metabolic and lysosomal storage diseases
P281 – P292	Ocular diseases
P293 – P308	Inflammatory and autoimmune diseases
P309 – P317	Immune responses in gene and cell therapy
P318 – P333	Insertional mutagenesis
P334 – P358	Cancer: oncolytic and suicide gene therapies
P359 – P393	Immunotherapy of cancer and infectious diseases

EXHIBITORS

Booth 1: Fanconi anaemia: see page 24

www.asoc-anemiafanconi.es

Booth 2: Oxford Genetics specialises in creating DNA plasmids and components that are fully inter-compatible. We provide a unique range of DNA products that make genetic engineering easier and more efficient, whilst also offering both custom cloning and DNA synthesis. Whatever your cloning requirements, we've got all bases covered.

http://oxfordgenetics.com

Booth 3: Sigma-Aldrich: see page 28

www.sigma-aldrich.com

Booth 4: BioReliance: see page 20

www.bioreliance.com

Booth 5: The Vector Core at the University of North Carolina, Chapel Hill (UNC Vector Core) operates as a full-service viral vector production organisation, producing nearly 1000 lots per year. We have extensive experience in vector design, process development, as well as manufacturing of research and clinical grade vectors to ensure your project is on time and on budget. Our goal is to deliver the highest quality vectors to our academic, government, foundation, and biotech industry clients. We also offer process development, assay development, and other support services to accelerate the research and development process.

http://genetherapy.unc.edu/jvl.htm

Booth 6: TrakCel is an orchestration platform to safeguard patients being treated with regenerative therapies. The platform ensures that any patient receives the most effective therapy at the right time and location. The platform efficiently tracks, traces and documents the movement and handling of multiple regenerative therapies. Using state of art technologies, the platform includes biometrics, Radio Frequency Identification (RFID) and Global Positioning. TrakCel is a comprehensive solution that covers all aspects of patient registration, sample collection, logistics, manufacturing, quality control. Real time data is generated and automatically monitored recording the location and the temperature of material in transit, issuing warnings and alarms if pre-defined limits are breached.

www.trakcel.com

EXHIBITORS

Booth 7: EUFETS, a German based company, supports the development and commercialisation of cell and gene therapies. Services include cGMP-compliant manufacturing of viral vectors, genetically modified cells and, as a new service, *in vitro* transcribed RNA. EUFETS offers process and assay development, validation, quality control, storage and QP release. EUFETS also supports preclinical product development (R&D/GLP studies) of biologics with customised *in vitro* bioanalytical programmes.

www.eufets.com

Booth 8: PeproTech was established in 1988 by a group of scientists who decided to focus their efforts on the development and production of recombinant cytokines for life-science research. Today, PeproTech is a world leader in supplying high quality cytokine products including E. coli, insect, and mammalian cell-derived recombinant proteins, their monoclonal/ polyclonal antibodies, ELI SA development kits, and other cytokine-related reagents.

www.peprotechec.com

Booth 9: ATMI: see page 20

www.atmi-lifesciences.com

Booth 10: Miltenyi Biotec: see page 21

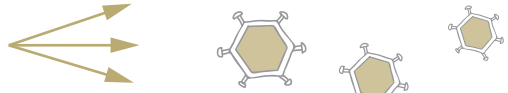
www.miltenyibiotec.com

Booth 11: The Andalusian Initiative for Advanced Therapies (IATA): see page 19

www.juntadeandalucia.es/terapiasavanzadas/en/central-office

Booth 12: NIMGenetics: see page 27

www.nimgenetics.com



EXHIBITORS

Booth 14: With a background of 30 years, Quality Assistance holds a unique position on the market for the development and validation of analytical methods. Thanks to our scientific expertise and six laboratories on one site (Mass Spectrometry, Physico-chemistry, Bioanalysis, Cell Culture, Molecular Biology and Microbiology), we assess your analytical development over the course of the cell expansion process for product characterisation, raw materials analysis, stability studies and bioanalysis. Our multi-disciplinary team, combined with the highest quality standards, ensures the best management of your projects, including troubleshooting and respect of timelines. It makes sense – and it is cost effective – to concentrate all analytical expertise on one site.

www.quality-assistance.com

Booth 15-16: GE Healthcare Life Sciences: see page 20

www.gelifesciences.com

Booth 17: Charles River is a full-service, global contract research organisation committed to delivering high-quality products and services to the pharmaceutical industry. Our broad range of capabilities spans each phase of discovery and development in every major therapeutic area, enabling you to enhance productivity and increase speed to market.

www.criver.com

Booth 18: Human Gene Therapy: see page 27

www.liebertpub.com/hum

Booth 20: Becton Dickinson: see page 16

www.bdbiosciences.com

EXHIBITORS

Booth 21: PlasmidFactory: see page 22

www.PlasmidFactory.com

Booth 22: Lonza offers world class technology platforms in the areas of GMP cell culture and viral-based therapeutic manufacturing, custom bio-therapeutic culture media, a large selection of primary and stem cells and a full line of custom bioassays. Our extensive experience in cell therapy process optimisation and scale-up innovation helps clients to safely and effectively advance their products through all phases of the commercial pipeline and maximise their return on investment. Our new viral-based therapeutics group provides viral vaccine manufacturing as well as viral vector mediated gene therapies. Our staff can design, develop, and implement a manufacturing process that meets your autologous or allogeneic therapeutic applications.

www.lonza.com

Booth 23: The Agencia Española de Medicamentos y Productos Sanitarios (AEMPS) is the body within the Spanish Ministry of Health, Social Services and Equality, which guarantees quality, safety, efficacy and accurate information on medicines and medical devices marketed in Spain. In its widest remit it covers research to end use, to protect and promote health in both human beings and animals. AEMPS' vision is to strengthen the sanitary authority of reference for citizens and healthcare professionals with regard to guarantees of quality, safety, efficacy, information and accessibility of medicines and medical devices.

www.aemps.gob.es

Booth 24: The Cellectis Group: see page 16

www.cellectis.com

EXHIBITORS

Booth 26: The Francisco de Vitoria University is a Catholic University that is part of an international network of sixteen universities and higher education institutions in Europe and America. The school of biosciences at Francisco de Vitoria University trains the next generation of biotechnology and health sciences professionals to respond to the challenges posed by today's society. We offer a biotechnology degree as well as an advanced therapies and biotechnological innovation master degree that aim to form innovative, critical, creative and inquisitive professionals who understand their science as a service to the society, with the objective to improve the quality of life. The training of both programs provides a solid theoretical, practical, technological and humanistic formation, which encourages personal and professional skill development, and includes practical training (placement) in national and international laboratories, hospitals, enterprises and research institutes.

www.ufv.es

Booth 28: GenoSafe: see page 21

www.genosafe.org

PROGRAMME

FRIDAY 25 OCTOBER 2013

EARLY PHASE CLINICAL TRIALS TRAINING COURSE *Pullman Hotel, Magna Room* *Chairs – Scientific Programme: Robin Ali, Alessandro Aiuti*	
09.00	Introduction, *Robin Ali and Alessandro Aiuti*
	INV005 Planning an academic trial – the sponsor's perspective *Kim Champion, University College London*
	INV006 Production of vector and genetically modified stem cells *Anne Galy, Généthon, Evry*
	INV007 The role of QP in assessing ATMPs *Eleanor Berrie, University of Oxford*
	INV008 AAV gene therapy for haemophilia *Amit Nathwani, Royal Free NHS Trust; UCL Cancer Institute; NHSBT*
	INV009 Lentiviral vector GT for beta-thalassemia *Giuliana Ferrari, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*
	INV010 Gammaretro and lentiviral vectors for the gene therapy of X-linked Chronic Granulomatous Disease *Manuel Grez, Institute for Biomedical Research, Georg-Speyer-Haus, Frankfurt*
11.30	Break
12.00	INV015 EU regulations for ATMP and clinical trials *TBC, EMA-CAT London*
	INV016 Ensuring GCP compliance, patient safety and data integrity *Kim Champion, University College London*
	INV017 Glybera® approval: a road map for advanced therapies in the orphan space *Harald Petry, Uniqure, Amsterdam*
	INV018 Phase Ib/IIa, escalating dose, single blind, clinical trial to assess the safety of the intravenous administration of expanded allogeneic adipose-derived mesenchymal stem cells (eASCs) to refractory rheumatoid arthritis (RA) patients *Lydia Dorrego, TiGenix, Madrid*
	INV019 Regulatory challenges in development of lentiviral *ex vivo* gene therapy products *Anne-Virginie Eggimann, BlueBirdBio Inc., Cambridge*
	INV020 ATMP in the EU; the long and winding road *Sol Ruiz, CAT, Spanish Medicines Agency (AEMPS), Madrid*
14.00	Lunch – exhibition and congress (at the Palacio Municipal de Congressos)

NOTES

I never said it was possible. I only said it was true. —Charles Richet

ciberer
CENTRO DE INVESTIGACIÓN BIOMÉDICA EN RED DE ENFERMEDADES RARAS

JORNADA DE DIVULGACIÓN CIBERER

25 de octubre,
Palacio Municipal de Congresos, Madrid
(Campo de las Naciones)

Terapias avanzadas para el tratamiento de Enfermedades Raras

Objetivo:

Acercar a los afectados por enfermedades raras, sus familias y el público en general los últimos avances en la aplicación clínica de las terapias avanzadas.

Programa:

9:15h	Inicio de acreditación y bienvenida
9:45h	**Bienvenida** *Dr. Antonio L. Andreu, Director del Instituto de Salud Carlos III* *Sra. Belén Crespo, Directora de la Agencia Española de Medicamentos y Productos Sanitarios*
10:00h	**Papel de la terapia génica en enfermedades raras** *Dra. Cristina Fillat*
10:30h	**Terapia génica en anemia de Fanconi** *Dr. Juan Bueren*
10:50h	**Terapia celular y génica epidermólisis bulosa** *Dra. Marcela del Río*
11:10h	**Terapia génica de porfirias hepáticas** *Dra. Gloria González-Aseguinolaza*
11:40h	Pausa Café
12:10h	**Terapia génica de mucopolisacaridosis** *Dra. Fátima Bosch*
12:30h	**Gene therapy of adrenoleukodistrophy** *Dra. Nathalie Cartier*
13:00h	**Gene therapy of immunodeficiencies** *Dra. Anne Galy*
13:30h	**Terapias avanzadas, la visión de los afectados** *D. Jordi Cruz* *Federación Española de Enfermedades Raras (FEDER)*
13:45h	**Conclusiones** *Dr. Francesc Palau. Director Científico del CIBERER*

Palacio Municipal de Congresos
Av. de la Capital de España Madrid, s/n 28042 Madrid
Teléfono: 00 34 91 722 04 00
www.madridec.com

Asistencia gratuita, necesario confirmar en: *eventos@ciberer.es*

ciberer · FEDER

regenerar

REGENER-AR is a European Consortium made with partners of private and academic institutions from five countries with the aim of developing a treatment for rheumatoid arthritis (RA) based on adipose mesenchymal stem cells (ASC).

The results from the phase IA/IIB clinical trial confirm the safety of treatment of RA with allogeneic ASC and support further clinical development.

Learn more about the REGENER-AR Project at

www.regenerar.eu

Bringing Regenerative Medicine into the Market:

Allogeneic eASCs Phase IB/ IIA clinical trial for treating Rheumatoid Arthritis

This project is supported by the 7th Framework Programme of the European Commission
EC-GA nº 279174

TiGenix · Inserm · TiGenix · Erasmus MC · University of Oxford · Ciemat · Mondiagon Assembly · Farma-Cros Ibérica · Sistemic · zabala

PROGRAMME

FRIDAY 25 OCTOBER 2013

EDUCATION DAY		
10.00	**Education session 1: Cancer gene therapy** *Sala Paris*	**Education session 2: Stem cells and tissue engineering** *Sala Berlin*
	INV001 Bases of gene therapy in leukemias *Chiara Bonini, San Raffaele Scientific Institute, Milan*	INV003 Neural stem cells *Josep Canals, Grupo de Células Madre y Medicina Regenerativa, Universidad de Barcelona*
	INV002 Recent developments in gene therapy of solid tumours *Rubén Hernández, Universidad de Navarra, Madrid*	INV004 Hepatic progenitor cells in liver regeneration *Stuart J. Forbes, University of Edinburgh*
11.30	Morning break	
12.00	**Education session 3: Gene therapy in rare diseases** *Sala Paris*	**Education session 4: From bench to bedside** *Sala Berlin*
	INV011 Translational research in the *ex vivo* gene therapy of monogenic diseases *Bobby Gaspar, UCL Institute of Child Health*	INV013 Clinical trials with mesenchymal stem cells in wound healing *Damián García-Olmo, Universidad Autónoma de Madrid*
	INV012 Progress and challenges of *in vivo* gene transfer with AAV vectors *Federico Mingozzi, Généthon, Evry; University Pierre and Marie Curie, Paris*	INV014 Ethic issues in cell and gene therapy *Odile Cohen-Haguenauer, Hôpital St-Louis, Univ Paris-Diderot, Sorbonne Paris-Cité & UMR 8113, ENS de Cachan*
13.30	Lunch – Exhibition and poster halls	

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

FRIDAY 25 OCTOBER 2013

MAIN CONFERENCE	
15.00	**Inaugural session – Auditorium** *Chairs: Luigi Naldini and Juan Bueren* Inaugural lecture: Gene therapy delivers *Luigi Naldini, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan*
15.45	**Plenary session 1: Cell reprogramming**
Auditorium	*Chair: Juan Carlos Izpisúa-Belmonte* INV022 Induction of toti-,pluri- and multipotency *Hans Schöler, Max Planck Institute for Molecular Biomedicine, Münster* INV023 Reprogramming *in vivo* is possible and generates a new type of iPS *Manuel Serrano, Spanish National Cancer Research Center (CNIO), Madrid* INV024 Human gene editing for stem cell based therapy *Juan Carlos Izpisúa-Belmonte, Salk Institute for Biological Studies, La Jolla, California; CMRB, Barcelona*
17.15	Afternoon break
17.45	**Parallel sessions 1a, 1b, 1c, 1d**
Auditorium	**1a: Transposon and non viral gene transfer** *Chairs: Zoltan Ivics, Laurence Cooper* INV025 The Sleeping Beauty transposon system for molecular medicine *Zoltan Ivics, Paul Ehrlich Institute, Langen* INV026 Non-viral CFTR gene delivery to the lungs of Cystic Fibrosis patients *Steve Hyde, University of Oxford* INV027 Clinical application of Sleeping Beauty system to engineer T-cell specificity *Laurence Cooper, MD Anderson Cancer Center, Houston* *Proffered papers* OR001 Collagen VII gene delivery via Sleeping Beauty transposon in COL7A1-deficient keratinocytes from epidermolysis bullosa patients *Maria Carmela Latella, Center for Regenerative Medicine, University of Modena; Reggio Emilia Modena Italy*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

FRIDAY 25 OCTOBER 2013

	OR002 An E3-14.7K peptide that promotes microtubules-mediated transport of plasmid DNA increases polyplexes transfection efficiency *Lucie Pigeon, Centre de Biophysique Moléculaire CNRS UPR4301 Inserm; University of Orléans* OR003 Non viral gene therapy clinical trial for pancreatic cancer *Pierre Cordelier, INSERM U1037, Toulouse*
Sala Madrid 17.45	**1b: Biology and manipulation of stem cells** *Chairs: Felipe Prosper, Josep Canals* INV028 Stemness control by cell cycle regulators *Isabel Fariñas, Departamento de Biología Celular and CIBERNED, Universidad de Valencia* INV029 X-Reactivation impacts human iPSC differentiation potential towards blood *Niels-Bjarne Woods, Lund's Stem Cell Center, Lund University* INV030 DLL4/Notch1 signaling is required for endothelial-to-hematopoietic transition in a hESC model of human embryonic hematopoiesis *Pablo Menéndez -Bujan, Gentro de genómica y Oncología de Granada (GENyO). Granada; Josep Carreras Leukaemia Research Institute; ICREA Research Professor, Barcelona* *Proffered papers* OR004 Improved manipulation of hematopoietic stem and progenitor cells (HSPC) for *ex vivo* gene therapy *Bernhard Gentner, San Raffaele Telethon Institute for Gene Therapy, Milan* OR005 Transient manipulation of haematopoietic stem cells with integrative deficient lentiviral vectors for improved cell expansion survival and engraftment *Steven Howe, University College London Institute of Child Health* OR006 Selective regulation of hematopoietic progenitors by estrogens as a basis for anti-leukemic strategies *Abel Sanchez-Aguilera, Spanish National Cardiovascular Center (CNIC), Madrid*
Sala Paris 17.45	**1c: Immune responses in gene and cell therapy** *Chairs: Federico Mingozzi, Hildegard Büning* INV031 Immune responses to AAV vectors in human trials *Federico Mingozzi, Généthon, Evry; University Pierre and Marie Curie, Paris* INV032 *In vitro* and *in vivo* use of lentiviral vectors to induce Ag-specific immune tolerance *Maria Grazia Roncarolo, San Raffaele Scientific Institute, Milan*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

FRIDAY 25 OCTOBER 2013

	INV033 Detection of nucleic acids by the innate immune system *Veit Hornung, Institut für Klinische Chemie und Pharmakologie, Universitätsklinikum Bonn* *Proffered papers* OR007 Accurate measurement of NAb status against AAV vector capsids and an approach toward managing its inhibitory effect *Hiroaki Mizukami, Jichi Medical University* OR008 Uncovering long-term survival and activity *in vivo* in humans of genetically engineered T memory stem cells by retroviral tagging *Serena Scala, HSR-TIGET, Milan* OR009 Small peptides blocking SR-A and SREC-I increase HDAd-mediated liver transduction through inhibition of Kupffer and liver sinusoidal endothelial cell uptake *Nicola Brunetti-Pierri, Telethon Institute of Genetics and Medicine, Naples*
Sala Berlin 17.45	**1d: Imaging in cell and gene therapy** *Chairs: Pilar Martín-Duque, Jesús Ruiz-Cabello* INV034 Multi-modal imaging of gene expression *in vivo* *Andreas H. Jacobs, European Institute for Molecular Imaging (EIMI) at the Westfalian Wilhelms University (WWU), Münster* INV035 Development of non viral gene delivery vectors through SPECT/CT imaging *Georges Vassaux, UMRE 4320 (CEA, Université de Nice, Centre Antoine Lacassagne)* INV036 Imaging of gene and cell therapy vectors *Iván Peñuelas, Department of Nuclear Medicine, University of Navarra, Pamplona* *Proffered papers* OR010 Somatotransgenic bioimaging: a novel biosensing platform for *in vivo* bioimaging *Tristan McKay, St. Georges University of London* OR011 *In vivo* imaging of mesenchymal stem cell recruitment into the tumor stroma of hepatocellular carcinoma (HCC) using a HIF-1α-specific sodium iodide symporter gene system *Kerstin Knoop, University Hospital of Munich* OR012 Thy1.1 p75NTR or CAR receptor targeting by lentiviral vectors leads to retrograde transport and transduction of spinal motor neurons following peripheral delivery *Nicholas Mazarakis, Imperial College London*
19.30	**Welcome reception**

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SATURDAY 26 OCTOBER 2013

MAIN CONFERENCE	
09.00	**Plenary session 2: Stem cells and stem cell niche**
Auditorium	*Chair: Stefan Karlsson* INV037 Neural crest contributions to the haematopoietic stem-cell niche *Simón Méndez, Centro Nacional de Investigaciones Cardiovasculares, Madrid* INV038 Hematopoiesis under the stress *Toshio Suda, Keio University, Tokyo* INV039 Regulation of normal and leukemic human stem cells: dynamic stem cell interactions with the bone marrow microenvironment *Tseve Lapidot, Weizmann Institute of Science, Rehovot*
10.30	Morning break
11.00	**Parallel sessions 2a, 2b, 2c, 2d**
Auditorium	**2a: Oncolytic viral therapy** *Chairs: Ramón Alemany, Len Seymour* INV040 Preclinical and early clinical development of an oncolytic group B adenovirus, ColoAd1 *Len Seymour, University of Oxford* INV041 Parvovirus infections: prospects for cancer treatment *Jean Rommelaere, German Cancer Research Center (DKFZ), Heidelberg* INV042 Clinical translation in oncolytic virotherapy *Kevin Harrington* INV043 Telomerase-targeting oncolytic adenovirus as thetherapeutic and diagnostic agent *Shunsuke Kagawa, Okayama University* *Proffered papers* OR013 ColoAd1 a group B oncolytic adenovirus: pre-clinical characterisation and development of 'armed' variants *Alice Brown, PsiOxus, Oxford* OR014 Results of a trial of compassionate use of Celyvir in children with metastatic and refractory solid tumors *Manuel Ramirez, Hospital Universitario Niño Jesús, Madrid* OR015 Pancreatic tumor targeting with cell type-specific miRNA-regulated oncolytic adenoviruses reduces local and systemic toxicity *Xavier Bofill De Ros, IDIBAPS/CIBERER*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SATURDAY 26 OCTOBER 2013

Sala Madrid 11.00	**2b: Primary immunodeficiencies** *Chairs: Alessandro Aiuti, Manuel Grez* INV044 Lentiviral gene therapy for primary immune deficiencies: preclinical evaluation of efficacy, safety and conditioning modalities *Gerard Wagemaker, Erasmus University Medical Center, Rotterdam* INV045 Thymic stromal pathologies: a target for gene replacement therapy? *George Holländer, University of Oxford; University of Basel* INV046 Current status and perspectives from international trials of gene therapy for primary immune deficiencies *Anne Galy, Généthon, Inserm U951, Evry* *Proffered papers* OR016 Development of new lentiviral vectors for the gene therapy of LAD-I *Diego León Rico, CIEMAT/CIBERER, Madrid* OR017 Efficient site-specific integration and *in situ* gene correction of human long-term repopulating hematopoietic stem cells by zinc finger nucleases *Pietro Genovese, San Raffaele Telethon Institute for Gene Therapy, Milan* OR018 Accumulation of proto-oncogene integrations triggering lymphoid as well as myeloid leukemia in WAS gamma-retroviral gene therapy *Anna Paruzynski, National Center for Tumor Diseases; German Cancer Research Center, Heidelberg*
Sala Paris 11.00	**2c: Muscular and bone diseases** *Chairs: Philippe Moullier, George Dickson* INV047 Forelimb treatment in a large cohort of dystrophic dogs supports delivery of a recombinant AAV for exon skipping in Duchenne patients *Philippe Moullier, UMR 1089 INSERM Translational Gene Therapy For Retinal And Neuromuscular Diseases, Institut de Recherche Thérapeutique 1, Université de Nantes* INV048 Optimised AAV-microdystrophin gene therapy for Duchenne muscular dystrophy *George Dickson, Royal Holloway University of London* INV049 Gene therapy of Myotubular Myopathy *Fulvio Mavilio, Généthon, Evry* *Proffered papers* OR019 MicroRNA-regulated cassettes aimed at preventing cardiac toxicity of CAPN3 gene transfer *Le Roy Florence, Généthon*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SATURDAY 26 OCTOBER 2013

	OR020 Gene transfer of TCIRG1 driven by clinically relevant promoters restores osteoclast function in Infantile Malignant Osteopetrosis *Ilana Moscatelli, Strategic Center for Stem Cell Biology, Lund* OR021 Insight into molecular events associated with partial restriction of Adeno-Associated Vector genome expression in dystrophic muscles *Jean-Baptiste Dupont, UMR INSERM 1089, Nantes*
Sala Berlin 11.00	**2d: Bioprocessing of cell and gene therapy products** *Chairs: Manuel Carrondo, Otto Merten* INV050 Production of lentiviral vectors by transfection of suspension cells in single use systems *Matthias Hebben, Généthon, Evry* INV051 Bioengineering approaches for up- and down- stream processing of human stem cells for clinical application *Paula Alves, ITQB, Universidade Nova de Lisboa; IBET, Oeiras* INV052 Challenges in vector and cell manufacturing for gene therapy *Paolo Rizzardi, MolMed, Milan* *Proffered papers* OR022 Development of the manufacturing process for the *ex vivo* gene therapy for ADA-SCID (GSK2696273) *Ekaterini Kotsopoulou, GlaxoSmithKline, Stevenage* OR023 Environmental risk assessment for the placing on the market of the gene therapy product Glybera *Ursula Jenal, Jenal & Partners Biosafety Consulting, Rheinfelden* OR024 Peptide-mediated engineering of 12 AAV serotypes *Dirk Grimm, Heidelberg University Hospital*
12.45	ESGCT General Assembly – Auditorium
12.45	Lunch – Exhibition and poster halls
14.15	**Plenary session 3: Gene and cell therapy in regenerative medicine**
Auditorium	*Chair: Fulvio Mavilio* INV053 MuStem cells: a therapeutic candidate for cell-based therapy of Duchenne Muscular Dystrophy *Yan Cherel and Karl Rouger, INRA, UMR 703, Oniris, Nantes* INV054 A clonal strategy for *ex vivo* gene therapy of epidermis *Yann Barrandon, Ecole Polytechnique Fédérale de Lausanne*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SATURDAY 26 OCTOBER 2012

	INV055 Systemic cell therapy for Epidermolysis Bullosa: repair of skin extracellular matrix *Jakub Tolar, University of Minnesota*
15.45	Afternoon break
16.15	**Parallel Sessions 3a, 3b, 3c, 3d**
Auditorium	**3a: Cell reprogramming** *Chairs: Angel Raya, Sarah Ferber* INV056 Modeling neurodegenerative disease through iPS cell technology *Angel Raya, Institute for Bioengineering of Catalonia (IBEC), Barcelona* INV057 Transdifferentiation of human adult cells into endocrine pancreatic cells; cell replacement therapy for diabetic patients *Sarah Ferber, Sheba Medical Centre, Tel Hashomer; Tel-Aviv University* INV058 AAV-mediated gene targeting approaches for the derivation of histocompatible pluripotent stem cells *David Russell, University of Washington* *Proffered papers* OR025 A possible therapeutic strategy for genetic diseases using hematopoietic stem cells generated from induced pluripotent stem cells *Makoto Otsu, University of Tokyo* OR026 Retrovirus insertion in iPSCs identifies genes facilitating somatic reprogramming *Ali Nowrouzi, National Center for Tumor Diseases (NCT); German Cancer Research Center (DKFZ), Heidelberg* OR027 Allele-preferred targeted correction of CFTRgene in Cystic Fibrosis induced pluripotent stem cells *Brian Davis, University of Texas*
Sala Berlin 16.15	**3b: Inflamatory and autoimmune diseases** *Chairs: David Klatzmann, Christian Jorgenssen* INV059 Immunoregulation without immunossupression: the promise of low-dose IL-2 *David Klatzmann, Pitié Salpêtrière Hospital, Paris* INV060 MSCs generate a $CD4^+CD25^+FOXP3^+$ regulatory T cell population *Christian Jorgenssen, Inserm U844, Hôpital saint-Eloi, Montpellier* INV061 Stem cell therapy for RA *Wilfried Dalemans, Tigenix, The Netherlands*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SATURDAY 26 OCTOBER 2013

	Proffered papers OR028 Disease-regulated local Interleukin-10 gene therapy diminishes synovitis and articular cartilage damage in experimental arthritis *Mathijs Broeren, Radboud University* OR029 Inflammatory effects of galectin-1-deficient regulatory T cells in a T cell transfer model of inflammatory bowel disease *Marina Garín, CIEMAT/CIBERER, Madrid* OR030 Single intravenous administration of viral vectors carrying IL23R: A promising approach for multiple sclerosis therapy *Miralles Consuegra, Universitat Autónoma de Barcelona*
Sala Paris 16.15	**3c: Aging and genetic instability syndromes** *Chairs: JakubTolar, María Blasco* INV062 Dyskeratosis congenita and related syndromes *Inderjeet Dokal, Queen Mary University of London* INV063 Telomeres as therapeutic targets for cancer and aging *María Blasco, Spanish National Cancer Research Centre (CNIO), Madrid* INV064 Towards the gene therapy of Fanconi anemia with lentiviral vectors *Juan Bueren, CIEMAT/CIBERER, Madrid* INV065 Approaches to the study of reprogramming-induced genomic instability in Fanconi anemia (FA) cells and the role of the FA pathway in early hematopoietic commitment *David Williams, Boston Children's Hospital, Harvard Medical School, Boston* *Proffered papers* OR031 Expression of a small internal fragment of Dyskerin decreases DNA damage and oxidative stress in Ataxia Telangiectasia cells *Rosario Perona, Instituto de Investigaciones Biomédicas CSIC/UAM; CIBER de Enfermedades Raras and IDIPaz, Madrid* OR032 Long-term preclinical studies of a FANCA lentiviral vector in a Fanconi anemia-A mouse model *Francisco Javier Molina-Estevez, CIEMAT/CIBERER, Madrid*
Sala Madrid 16.15	**3d: Immunotherapy of cancer** *Chair: Barbara Savoldo, Chiara Bonini* INV066 TCR gene editing for the treatment of hematological malignancies *Chiara Bonini, San Raffaele Scientific Institute, Milan*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SATURDAY 26 OCTOBER 2013

	Proffered papers OR033 Towards clinical γδTCR gene therapy: the optimal γδTCR T cell product *Sabine Heijhuurs, UMC Utrecht* OR034 Adoptively transferred TCR gene-transduced lymphocytes persist with anti-tumor reactivity in patients with MAGE-A4+ esophageal cancer *Hiroaki Ikedda, Mie University, Japan* OR070 Entry targeted gene transfer into functional subpopulations of human T cells *Katharina Uhlig, Paul-Ehrlich-Institut, Langen* OR036 A new fully humanised transgenic mouse model for predicting the hematological toxicities of CD44v6-CAR T cells *Attilio Bondanza, San Raffaele Hospital Scientific Institute* OR037 A versatile pre-clinical *in vivo* model to evaluate the efficacy of T cell receptor gene therapy *Wolfgang Uckert, Max-Delbrăck Center for Molecular Medicine, Berlin*
18.00 18.30	Poster session A (Odd numbers) – Poster halls Presented posters session A – see table on page 68 for details
21.00	Speakers' Dinner – by invitation only *Coaches will depart from Pullman Hotel at 20.15*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SATURDAY 26 OCTOBER 2013

PRESENTED POSTERS SESSION A

Nº	TIME	PRESENTER	TITLE	ITINERARY
P001	18:30	Nina Harmening	Use of Sleeping Beauty transposase mRNA for safe and efficient gene delivery in pigment epithelial cells	Itinerary 1A: NON VIRAL VECTORS / NANOTECHNOLOGY AND RNA THERAPIES / VIRAL VECTORS DEVELOPMENTS Coordinator: Mª Concepción Tros
P003	18:35	Mª Concepción Tros	Folate-polycationic amphiphilic cyclodextrin-DNA nanocomplexes for gene delivery *in vitro* and *in vivo*	
P005	18:40	Teerapong Yata	Smart bacteriophage nanocomplex: a hybrid multi-component particle for safe and efficient gene transfer	
P007	18:45	Robert Carlisle	Increased Adenovirus density for increased ultrasound-mediated delivery to tumors	
P043	18:50	Peter Kurre	Lentiviral episomes provide long-term transgene expression in dividing cells	
P045	18:55	Mania Ackermann	Tightly regulated Doxycycline (Dox)-inducible lentiviral vectors for human myeloprotective gene therapy: *in vitro* and CD34+- xenotransplant studies	
P047	19:00	Rasmus Bak	DNA transposition by protein transduction of the piggyBac transposase from lentiviral Gag precursors	
P049	19:05	Julia Debora Suerth	Alpharetroviral SIN vectors for T-cell based therapy	
P079	18:30	Claudio Mussolino	TALENs mediate genome editing with superior specificity and lower toxicity than matched ZFNs	Itinerary 2A: GENE AND CELL TARGETING / STEM CELLS BIOLOGY Coordinator: Claudio Mussolino
P081	18:35	Paula Río	Generation of disease free hematopoietic progenitors from FA patient cells using a combined gene targeting and cell reprogramming strategy.	
P083	18:40	Xavier Anguela	ZFN mediated targeting of albumin: a platform for expression of multiple therapeutic genes *in vivo*	
P085	18:45	Araksya Izmiryan	Meganuclease-based therapy for recessive dystrophic epidermolysis bullosa	
P105	18:50	África González-Murillo	A role for NIK (NFkB Inducing Kinase) in the response of hematopoietic stem cells to stress	
P107	18:55	Jose Manuel Morante-Redolat	Transcriptional repression of Bmp2 by cell cycle inhibitor p21 links quiescence to neural stem cell maintenance in the subependymal niche	
P109	19:00	Javier García-Castro	Novel *in vivo* method to test the differentiation potential of mesenchymal progenitor cell cultures	

SATURDAY 26 OCTOBER 2013

PRESENTED POSTERS SESSION A

P155	18:30	Antonia Follenzi	iPSC-based strategy to correct the bleeding phenotype in Haemophilia A	Itinerary 3A: CELL REPROGRAMMING / LYMPHO-HEMATOPOIETIC GENE THERAPY Coordinator: Guillermo Guenechea
P157	18:35	Maria Garcia-Bravo	Development of a liver-specific marker transgenic mouse for the study of alternative pathway of hepatic regeneration from bone marrow cells	
P171	18:40	Scaramuzza	Persistent multilineage engraftment and WASP expression after lentiviral mediated CD34+ cell gene therapy for the treatment of Wiskott-Aldrich Syndrome	
P173	18:45	Sabine Charrier	Biosafety studies of a clinically-applicable lentiviral vector for gene therapy of RS-SCID.	
P177	18:50	Olivier Negre	Long term safety of clinical grade LentiGlobin vectors in β-thalassemic and normal mice	
P179	18:55	María García-Gómez	Lentiviral-based gene therapy for Pyruvate Kinase deficiency	
P183	18:30	Agnieszka Jazwa	Molecular changes and gene therapy of the infarcted myocardium	Itinerary 4A: CARDIOVASCULAR DISEASES / NEURO-MUSCULAR DISEASES Coordinator: Nathalie Cartier
P185	18:35	Anne-Catherine Prats	IRES-based vectors for a combined gene therapy of heart ischemia.	
P187	18:40	Sebastien Verhenne	Correction of murine ADAMTS13 deficiency using the 'Sleeping Beauty' transposon system	
P197	18:45	Vasco Meneghini	Intracerebral administration of lentiviral vectors in juvenile non-human primates: a biodistribution study	
P199	18:50	Woo Jin Park	Cleavage of amyloid-β by the Nuclear Inclusion a (NIa) protease of turnip mosaic virus	
P201	18:55	Laurence Dubreil	Selective neuronal targeting by self-complementary AAV9 or AAV10 via intrathecal delivery in nonhuman primate: application for chronic pain or motor neurons diseases.	
P203	19:00	Maria Grazia Biferi	Recombinant AAV9 vectors to silence the mutant SOD1 gene in Amyotrophic Lateral Sclerosis	
P205	19:05	Laurine Buscara	miRNA-mediated restriction of myotubularin expression to the skeletal muscle corrects pathology and prevents cardiac toxicity in a murine model of X-linked myotubular myopathy	

PROGRAMME

SATURDAY 26 OCTOBER 2013

PRESENTED POSTERS SESSION A				
P229	18:30	Fernando de Miguel	*In vitro* effects of hyaluronate on adipose tissue-derived mesenchymal stem cells	Itinerary 5A: BONE, OTHER DISEASES AND AGEING / SKIN DISEASES / METABOLIC AND LYSOSOMAL STORAGE DISEASES Coordinator: Fernando Larcher
P231	18:35	Daniel Amat-Trujillo	Study of the osteoconductive and angiogenic potential of Electron Beam Melting Titanium (EBMT)	
P245	18:40	Clemens Hättner	Double RNA trans-splicing induced gene repair of COL7A1	
P247	18:45	Marta Carretero	Development of bioengineered skin-humanised mouse models for inflammatory skin diseases	
P261	18:50	Esperanza Lopez-Franco	Preliminary safety data from phase I clinical trial in acute intermittent porphyria	
P263	18:55	Ilaria Visigalli	*In vivo* AAV-mediated genetic engineering of white and brown adipose tissue in adult mice	
P265	19:00	Verónica Jiménez-Cenzano	Multigenic lentiviral vectors for anti-angiogenic treatment of age-related macular degeneration (AMD)	
P293	18:30	Jonathan Finn	Modulation of ATP:adenosine balance by exosome and RAIN (Recombinant Anti-Inflammatory fusioN) delivery of CD39 and CD73 iseffective in reducing pro-inflammatory cytokine and chemokine production	Itinerary 6A: INFLAMMATORY AND AUTOIMMUNE DISEASES / INSERTIONAL MUTAGENESIS Coordinator: Sarah Ferber
P295	18:35	Mathijs Broeren	The TSG-6 promoter is a promising candidate for disease-induced expression of biological drugs in the joints of rheumatoid arthritis patients	
P319	18:40	Stefano Annunziato	Identification of genes involved in the resistance to targeted anti-cancer therapies by lentiviral vector-based insertional mutagenesis	
P321	18:45	Michael Rothe	*In vivo* gentoxicity profile of a chimeric promoter driven lentiviral vector for X-CGD in mice	
P323	18:50	Arianna Moiani	Self-inactivating alpharetroviral vectors have a low genotoxic integration profile in human CD34+ hematopoietic progenitor cells	
P325	18:55	Kerstin Cornils	"Coloured" barcodes for clonal tracking	

SATURDAY 26 OCTOBER 2013

PRESENTED POSTERS SESSION A				
P335	18:30	Eneko Villanueva	Tissue-specific regulation of adenoviruses by incorporation of miR target sites controlling the late-phasefiber protein	Itinerary 7A: CANCER: ONCOLYTIC AND SUICIDE GENE THERAPIES / IMMUNOTHERAPY OF CANCER AND INFECTIOUS DISEASES Coordinator: Len Seymour
P337	18:35	Ramón Alemany	iRGD (tumor-penetrating peptide)-modified oncolytic adenovirus shows enhanced antitumor efficacy	
P359	18:40	Attilio Bondanza	Off-tumor expression of the target antigen does not predict CAR-T cell killing: a foundation for the safety of CD44v6-targeted T cells	
P361	18:45	Simona Bramante	Serotype 5/3 chimeric oncolytic adenovirus coding for GM-CSF for treatment of melanoma: results *in vitro* in rodents and humans.	
P363	18:50	Peng Huang	Immunomodulation and anti-cancer activity of REIC/Dkk-3 protein	
P365	18:55	Christian Smerdou	Short-term intratumoral IL-12 expressed from an alphaviral vector is sufficient to induce an efficient antitumoral response against spontaneous hepatocellular carcinomas	

PROGRAMME

SUNDAY 27 OCTOBER 2013

08.45	**Plenary session 4: Hematopoietic stem cell gene therapy**
Auditorium	*Chair: Nathalie Cartier* INV067 Clinical and biological results from the Paris GT clinical trials: what did we learn?" *Marina Cavazzana, Hôpital Universitaire Necker, Paris* INV068 Gene therapy for primary immunodeficiencies *Adrian Thrasher, University College London Institute of Child Health* INV069 Gene therapy for Wiskott-Aldrich Syndrome *Alessandro Aiuti, San Raffaele Telethon Institute for Gene Therapy, Milano* INV070 Clinical trial of HSC gene therapy for Metachromatic Leukodystrophy *Alessandra Biffi, San Raffaele Telethon Institute for Gene Therapy, Milano* INV071 Hematopoietic stem cell gene therapy with lentiviral vector in 4 patients with cerebral X-linked adrenoleukodystrophy: long-term outcome and comparison of efficacy with allogeneic hematopoietic stem cell transplantation. *Patrick Aubourg, INSERM UM745, University Paris-Descartes, Hôpital Bicêtre-Paris Sud*
10.45	Morning break
11.15	**Parallel sessions 4a, 4b, 4c, 4d**
Sala Madrid	**4a: Red blood cell diseases** *Chairs: Giuliana Ferrari, José C. Segovia* INV072 Gene therapy for Thalassemia: the challenge of a new cure *Giuliana Ferrari, HSR TIGET, San Raffaele Telethon Institute for Gene Therapy, Milan* INV073 Advanced therapies for the treatment of erythroid metabolic diseases: the Pyruvate Kinase deficiency *José C. Segovia, CIEMAT/CIBERER, Madrid* INV074 Gene therapy for sickle cell disease *Donald Kohn, University of California, Los Angeles ; Sangamo Biosciences Inc., Richmond, California* *Proffered papers* OR038 Zinc finger nucleases targeting the beta-globin locus drive efficient correction of the sickle mutation in CD34+ cells *Megan Hoban, University of California, Los Angeles*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SUNDAY 27 OCTOBER 2013

	OR039 G-CSF+plerixafor results in successful remobilisation and single apheresis collections in thalassemic patients after primary mobilisation failure *Evangelia Yannaki, George Papanicolaou Hospital, Thessaloniki* OR040 Parallel assessment of a globin lentiviral vector after transduction of IPs and somatic hematopoietic stem cells from the same transplanted human β-thalassemia patient *Leila Maouche-Chretien, CEA Institute of Emerging Diseases and Innovative Therapies (iMETI), Fontenay aux Roses*
Sala Paris 11.15	**4b: Cardiovascular diseases** *Chairs: Seppo Ylä-Herttuala, Antonio Bernad* INV075 VEGF gene therapy in ischemic myocardium *Seppo Ylä-Herttuala, University of Eastern Finland* INV076 Drug and cell delivery systems for cardiac repair *Felipe Prosper, Universidad de Navarra, Pamplona* INV077 Adult cardiac stem cells in mammalian heart homeostasis and repair are defined by Bmi1 expression *Antonio Bernad, CNIC (ISCIII), CNB-CSIC, Madrid* *Proffered papers* OR041 Human cardiac progenitor cells "Tolerogenic/Modulator" immune behavior designates them as low-risk high-benefit allogenic cardiac repair cells *Reem Al-Daccak, INSERM UMRS940 and AP-HP, Paris* OR042 Vascular endothelial and hepatocyte growth factor gene therapy in patients with critical limb ischemia *Andrei Anghel, University of Medicine and Pharmacy Victor Babes, Timisoara* OR043 Combinatorial RNAi- and receptor-based therapy against coxsackievirus B3 excerts additive antiviral effects *in vitro* and *in vivo* *Elisabeth Stein, Technische Universität, Berlin*
Auditorium 11.15	**4c: Neural diseases** *Chairs: Alessandra Biffi, José L. Barneo* INV078 GDNF-based cell therapy in Parkinson's disease *José López-Barneo, Universidad de Sevilla/CIBERNED* INV079 Gene therapy for neurodegenerative diseases: strategies for efficient targeting of the central nervous system *Nathalie Cartier, Université Paris Sud and INSERM U986 / CEA MIRcen*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SUNDAY 27 OCTOBER 2013

	INV080 Intra cerebral administration of AAVrh.10 carrying human SGSH and SUMF1 cDNAs in children with MPSIIIA disease: result of a phase I/II trial *Marc Tardieu, Université Paris Sud; INSERM U1012* *Proffered papers* OR044 AAV vectors produced by a GMP-compliant and scalable production platform mediate a CNS delivery platform using MRI-guided convection enhanced diffusion in NHP *Bas Blits, UniQure BV, Amsterdam* OR045 A soluble form of wild-type Tau protein is transported in rat brain through a trans-synaptic mechanism: implications for Tau spreading in sporadic Tauopathies *Nicole Déglon, Lausanne University Hospital (CHUV)* OR046 Bone marrow mononuclear cell therapy for amyotrophic lateral sclerosis. Preliminary results of a randomised, double-blind, stratified controlled, parallel group phase I-II clinical trial *Miguel Blanquer, University Hospital Virgen de la Arrixaca; Murcia University*
Sala Berlin 11.15	**4d: Nanotechnology and RNA therapeutics** *Chairs: Jesús M. de la Fuente, Juan C. Ramírez* INV081 Reduction sensitive nanogels from side chain functional polyglycidols as non-viral transfection systems *Jurgen Groll* INV082 Designing gold nanoparticles for *in vivo* gene silencing as a new therapeutic tool *Jesús M. de la Fuente, University of Zaragoza* INV083 Advanced technologies for engineering functional cardiac tissues *Tal Dvir, Tel Aviv University* *Proffered papers* OR047 Therapeutic efficacy of a systemically delivered oncolytic adenovirus – Biodegradable polymer complex *Chae-Ok Yun, University of Utah, Salt Lake City* OR048 MicroRNA inhibition by dual-targeting and clustered tough decoy inhibitors *Anne Kruse Hollensen, University of Aarhus, Denmark* OR049 Robust RNAi enhancement via human Argonaute-2 overexpression from plasmids viral vectors or cell lines *Dirk Grimm, Heidelberg University Hospital*
13.00	SETGyC General Assembly – Auditorium
13.00	Lunch – Exhibiton and poster halls

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SUNDAY 27 OCTOBER 2013

14.30	**Plenary session 5: Gene and cell therapy for cancer**
Auditorium	*Chair: Len Seymour* INV084 Engineered T cells for cancer therapy *Carl June, University of Pennsylvania, Philadelphia* INV085 Of CARs and TRUCKs: Chimeric antigen receptor (CAR) redirected T cells with inducible IL-12 *Heinrich Abken, University of Cologne* INV086 Adoptive immunotherapy with CAR-modified T cells *Gianpietro Dotti, Baylor College of Medicine, Houston, Texas*
16.00	Afternoon break
16.30	**Parallel sessions 5a, 5b, 5c, 5d**
Auditorium	**5a: Blood coagulation diseases** *Chairs: Katherine High, Thierry VandenDriessche* INV087 A phase I safety study in subjects with severe Hemophilia B using a single-stranded AAV8 vector to deliver the gene for factor IX *Katherine High, Children's Hospital of Philadelphia* INV088 AAV-mediated gene transfer in patients with severe Hemophilia B: longer follow-up and expansion of the high dose cohort *Amit C. Nathwani, Royal Free NHS Trust; UCL Cancer Institute; NHSBT* INV089 Overcoming the challenges in gene therapy for haemophilia *Thierry VandenDriessche, Free University of Brussels; University of Leuven* INV021 Design AAV vectors for clinical success in Hemophilia *Jude Samulski, University of North Carolina, Chapel Hill* *Proffered papers* OR050 Transcriptional and post-transcriptional targeting of FVIII expression to overcome immunological responses to gene therapy for Hemophilia A *Antonia Follenzi, University of Piemonte Orientale, Novara, Italy* OR051 Liver gene therapy by lentiviral vectors provides stable clinical benefit in three hemophilia B dogs and eradicates factor IX inhibitors in mice *Alessio Cantore, San Raffaele Telethon Institute for Gene Therapy, Milan*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SUNDAY 27 OCTOBER 2013

Sala Paris 16.30	**5b: Regenerative medicine** *Chairs: Javier García-Sancho, José María Moraleda* INV090 Fucosylated mesenchymal stem cells to treat systemic bone diseases *José María Moraleda, University of Murcia* INV091 Engineering of hydrogel-based bioinks for the fabrication of cell-laden 3D constructs *Jos Malda, Utrecht University* INV092 Bone regeneration based on tissue engineering conceptions – a 21st century perspective *Dietmar Hutmacher, Queensland University of Technology* *Proffered papers* OR052 Mesenchymal stromal cells reduce graft failure in autologous transplantation models with a high risk of poor engraftment *María Fernández García, CIEMAT/CIBERER, Madrid* OR053 Combining tissue engineering with metal scaffolds in orthopaedics to improve osteointegration of endoprostheses *Leonor Santos Ruiz, University of Málaga/CIBER-BBN* OR054 Aerosol-based cell therapy as a new strategy for treatment of airway injury *Badrul Hisham Yahaya, Universiti Sains, Malaysia*
Sala Madrid 16.30	**5c: Gene editing** *Chairs: Toni Cathomen, Rafael Yañez* INV093 Genome editing with zinc finger nucleases *Mike Holmes, Sangamo BioSciences, Inc., Richmond, California* INV094 Targeted transgene integration in human hematopoietic stem cells and induced pluripotent stem cells from normal donors and SCID-X1 patients *Angelo Lombardo, San Raffaele Telethon Institute for Gene Therapy; San Raffaele Scientific Institute; San Raffaele Hospital, Milan* INV095 Targeted gene editing in OPSC disease models *Toni Cathomen, University Medical Center Freiburg* *Proffered papers* OR055 Gene knock-in using specific TALE nucleases restores erythroid differentiation defect of human iPSC obtained from pyruvate kinase deficient patients *Zita Garate, CIEMAT/CIBERER, Madrid*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SUNDAY 27 OCTOBER 2013

	OR056 Rescue of T-cell deficiency in Prkdc scid mice by transplantation of gene-edited haematopoietic stem cells *Rafael Yáñez, Royal Holloway University of London* OR057 *In vivo* gene repair of inherited liver diseases using artificial endonucleases: application to Crigler Najjar disease *Cecilia Abarrategui Pontes, UMR 1064 University Hospital, Nantes*
Sala Berlin 16.30	**5d: Skin diseases** *Chairs: Marcela del Río, Alain Hovnanian* INV096 Protein, cell and gene therapies for rare skin disorders *Marcela del Río, CIEMAT/CIBERER; Universidad Carlos III de Madrid* INV097 Gene therapy for recessive dystrophic epidermolysis bullosa: the GENEGRAFT European project and beyond *Alain Hovnanian, INSERM U781; Necker hospital; University Paris Descartes* INV098 *In vivo* mobilisation of bone marrow mesenchymal stem cells accelerates cutaneous regeneration in epidermolysis bullosa *Katsuto Tamai, Osaka University* *Proffered papers* OR058 Lentiviral-mediated COL7A1 gene modified autologous cell therapy for Recessive Dystrophic Epidermolysis Bullosa (RDEB) *Farhatullah Syed, Institute of Child Health Molecular Immunology Unit UCL* OR059 Skin electroporation of a plasmid encoding hCAP-18/LL-37 host defense peptide promotes the healing of non-diabetic diabetic and ischemic wounds *Gaelle Vandermeulen, Université Catholique de Louvain, Brussels* OR060 Allogenic bone marrow derived mesenchymal stem cells for the treatment of Dystrophic Epidermolysis Bullosa *Hala Gabr, Faculty of Medicine, Cairo University*
18.15 18.30	Poster session B (Even numbers) – Poster halls Presented posters session B – see table on page 84 for details
21.00	Gala dinner – Casino de Madrid, Alcalá 15, 28014 Madrid (Metro Sevilla) *Coaches will depart from front of Palacio Municipal at 20.15*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

SUNDAY 27 OCTOBER 2013

PRESENTED POSTERS SESSION B

Nº	TIME	PRESENTER	TITLE	ITINERARY
P002	18:30	Virginie Escriou	Efficient and non-toxic siRNA delivery vector for systemic administration	Itinerary 1B: NON VIRAL VECTORS / NANOTECHNOLOGY AND RNA THERAPIES / VIRAL VECTORS DEVELOPMENTS Coordinator: Pilar Martín-Duque
P004	18:35	Musa Marimani	Inhibition of hepatitis B virus replication using guanidinopropyl modified siRNAs	
P006	18:40	Mariana Conceicao	Targeted lipid-based strategies to silence Machado-Joseph disease through the systemic route	
P044	18:45	Philip Boehme	Towards a direct comparison of HC-AdV vectors utilizing different genetic elements for stable transgene expression	
P046	18:50	Lester Suarez	The role of Furin in recombinant Adeno-Associated Viral vectors infection and transduction	
P048	18:55	Fedor Svinartchouk	Serum proteins impact efficiency of rAAV vectors in a species-specific manner	
P050	19:00	Michiko Tanaka	The efficacy and safety of DVC1-0101 for intermittent claudication secondary to peripheral artery disease: study protocol of a randomised phase IIb trial	
P080	18:30	Pilar Muñoz-Fernandez	Development of ZFN-based pre-clinical *in vitro* cell model in human embryonic stem cells for Wiskott-Aldrich syndrome	Itinerary 2B: GENE AND CELL TARGETING / BIOPROCESSING, MANUFACTURING AND REGULATORY ISSUES Coordinator: Francisco Martín-Molina
P082	18:35	Nicolisha Narainpersad	Mitochondrial gene targeting in mammalian systems using novel 'mitochondriotropic' liposomes	
P084	18:40	Els Verhoeyen	Baboon retrovirus envelope pseudotyped LVs allow efficient transduction of progenitor T cells thymocytes and adult T and B cells	
P124	18:45	Ana S. Coroadinha	Single step cloning-titration method: accelerating the development and engineering of high-titer retro and lentiviral vector producer cell lines	
P126	18:50	Rainer Loew	HEK293-platform for thegeneration of clinical-grade gamma-retroviral vectors	
P128	18:55	Florian Sonntag	Setting up a transfection-based rAAV production process in the Integrity iCellis™ single use fixed-bed bioreactor	
P130	19:00	Beatriz Fernandez-Munoz	Xeno-free isolation and expansion of GMP fibroblasts for cell therapy	

SUNDAY 27 OCTOBER 2013

PRESENTED POSTERS SESSION B

P182	18:30	Michiko Tanaka	A pilot study of quality of life for patients with chronic critical limb ischemia after gene therapy	Itinerary 3B: CARDIOVASCULAR DISEASES / NEURO-MUSCULAR DISEASES Coordinator: Seppo Ylä-Herttuala
P184	18:35	Javier García	Comparison of intrapericardial versus intracoronary delivery of Mesenchymal Stem Cells in a swine model of myocardial infarct	
P186	18:40	Urszula Florczyk	Non-inflammatory angiogenesis is impaired while the inflammation-driven revascularisation of ischemic tissue is accelerated by the lack of Nrf2	
P198	18:45	Javier Villadiego	Protection and repair of the dopaminergic nigrostriatal pathway by striatal xenografts of *in vitro* expanded CB cells in a chronic Parkinson's disease model	
P200	18:50	Jonathan Jones	Bonemarrow-derived mesenchymal stem cells protect degenerating cells in the dorsalroot ganglia of ataxic mice: a step forward towards a clinical application	
P202	18:55	Rajvinder Karda	Somatic transgenesis of the central nervous system for disease modelling and treatment	
P228	18:30	Yolanda Menenedez	*In vitro* effects of hyaluronate on adipose tissue-derived mesenchymal stem cells	Itinerary 4B : BONE, OTHER DISEASES AND AGEING / SKIN DISEASES / METABOLIC AND LYSOSOMAL STORAGE DISEASES Coordinator: Hildegard Büning
P230	18:35	Arantza Infante	Unexpected autophagy activation in a model of premature aging based on hMSCs	
P246	18:40	Sergio Crespo Garcia	Fate and function of adipose-derived mesenchymal stem cells (ADMSC) in bioengineered skin equivalents *in vitro* and *in vivo*	
P248	18:45	Ulrich Koller	A screening system accelerates the design of RNA trans-splicing molecules for skin cancer therapy	
P260	18:50	Marta Swierczek	Non-viral gene delivery by Sleeping Beauty transposon vectors for gene therapy of Gaucher disease	
P262	18:55	Giulia Bortolussi	Life-long liver-specific AAV-mediated gene therapy in a Crigler-Najjar mouse model	

PROGRAMME

SUNDAY 27 OCTOBER 2013

PRESENTED POSTERS SESSION B				
P282	18:30	Anne Louise Askou	Multigenic lentiviral vectors for anti-angiogenic treatment of age-related macular degeneration (AMD)	Itinerary 5B: OCULAR DISEASES / IMMUNE RESPONSES IN GENE AND CELL THERAPY Coordinator: Robin Ali
P284	18:35	Sofia Calado	pEPito-based vectors enable long-term expression in mouse models of diabetic retinopathy	
P286	18:40	Sonia Simao	Glucose and H2O2 modulate the expression of VEGF isoforms in human retinal cells	
P294	18:45	Sílvia Casacuberta	Antigen-specific myeloid-derived suppressor cells generated during retroviral transduction of murine bone marrow ameliorate experimental autoimmune encephalomyelitis	
P310	18:50	Rik Gijsbers	Immunological ignorance allows long-term gene expression following perinatal rAAV-mediated gene transfer to murine airways	
P312	18:55	Debbie Le Blon	Expression of the M2-type cytokines IL4 and IL13 prevents mesenchymal stem cell graft infiltration by microglia/macrophages in the central nervous system of immune competent mice	
P334	18:30	Yeon-Soo Kim	Therapeutic gene delivery to glioblastomas by enhanced retroviral vectors eradicate brain tumors and promotes survival	Itinerary 6B: CANCER: ONCOLYTIC AND SUICIDE GENE THERAPIES / IMMUNOTHERAPY OF CANCER AND INFECTIOUS DISEASES Coordinator: Renata Stripecke
P336	18:35	Haruki Kaku	Advanced two-step transcriptional amplification as a novel method for cancer-specific gene expression and imaging	
P338	18:40	Jung-Joon Min	Bacterium-based microrobot for visualisation of tumor targeting and drug delivery	
P360	18:45	Cecile BAUCHE	Development and preclinical evaluation of a lentiviral based anti-HIV therapeutic vaccine	
P362	18:50	Estanislao Nistal-Villan	Adeno-associated virus vectors expressing IFN-β induction pathway activating elements as alternative antiviral treatment	
P364	18:55	Dirk Grimm	Short-term intratumoral IL-12 expressed from an alphaviral vector is sufficient to induce an efficient antitumoral response against spontaneous hepatocellular carcinomas	

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

MONDAY 28 OCTOBER 2013

09.15	**Parallel sessions 6a, 6b, 6c, 6d**
Sala Madrid	**6a: Metabolic and lysosomal storage diseases** *Chairs: Jesús Prieto, Thierry VandenDriessche* INV099 Towards a gene therapy for neurological and somatic MPSIIIA *Fátima Bosch, Universitat Autònoma de Barcelona* INV100 Gene therapy for acute intermitent porphyria *Gloria González-Aseguinolaza, CIMA, Pamplona* INV101 Gene Therapy of Mucopolysaccharidosis VI *Alberto Auricchio, TIGEM; Federico II University, Naples* *Proffered papers* OR061 Efficacy of combined gene/cell therapy in a murine model of Globoid cell Leukodystrophy *Alessandra Ricca, San Raffaele Scientific Institute, Milan* OR062 GeneTherapy using an AAV2/8 vector corrects the biochemical imbalances in a murine model of MNGIE *Javier Torres Torronteras, Vall d'Hebron Institut de Recerca; CIBERER, Barcelona* OR063 Evaluation of intrathecal rAAV vectors in canine mucopolysaccharidosis VII *Mark Haskins, University of Pennsylvania, Philadelphia*
Sala Berlin 09.15	**6b: Ocular diseases** *Chairs: Robin Ali, John Flannary* INV102 Effective transplantation of photoreceptors derived from three-dimensional cultures of embryonic stem cells *Robin Ali, UCL Institute of Ophthalmology, London* INV103 Eye diseases *John Flannary, University of California, Berkeley* INV104 Transposon-mediated transfection of iris and retinal pigment epithelial *Gabrielle Thumann, Hôpitaux Universitaires de Genève* *Proffered papers* OR064 Hyperglycemia and oxygen tension control the expression of the glucose transporter GLUT-1 in models of Diabetic Retinopathy *Sofia Calado, University of Algarve, Faro*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

MONDAY 28 OCTOBER 2013

	OR065 Phase I clinical study of a third-generation Simian Immunodeficiency Virus (SIV)-based lentiviral vector carrying human Pigment Epithelium-Derived Factor (PEDF) gene for patients with retinitis pigmentosa *Yasuhiro Ikeda, Kyushu University, Fukuoka* OR066 Simple generation of self-organising neural retina-like structures from human iPS cells *Sacha Reichman, INSERM UMR_S968 CNRS UMR 7210 Université Pierre et Marie Curie, Paris*
Auditorium 09.15	**6c: Immunotherapy of cancer (II) and infectious diseases** *Chair: Renata Stripecke* INV105 High-throughput identification of T cell receptor (TCR) genes with TCR gene capture *Carsten Linnemann, The Netherlands Cancer Institute, Amsterdam* INV106 Adaptive DC immunotherapy after hematopoietic cell therapy *Renata Stripecke, Hannover Medical School* *Proffered papers* OR067 DAI-armed double deleted oncolytic vaccinia virus displays enhanced anti-tumor activity by eliciting a more robust anti-tumor immune response *Cristian Capasso, University of Helsinki* OR068 αβ-T cell receptor-based gene therapy targeting the common tumor-antigen survivin *Barbara Savoldo, Baylor College of Medicine, Houston* OR069 Infusion of ZFN CCR5 modified CD4 T-cells (SB-728-T) led to long term reconstitution of CD4 T-cells and reduction of HIV-DNA levels in HIV infected subjects on ART *Dale Ando, Sangamo BioSciences Inc., Richmond, California* OR035 T-cell engineering for "off-the-shelf" adoptive immunotherapy *Laurent Poirot, Cellectis Therapeutics*
Sala Paris 09.15	**6d: Viral vector developments** *Chairs: Axel Schambach, Marinee Chuah* INV107 Adeno-Associated Virus (AAV) vectorology on the move *Hildegard Büning, University of Cologne* INV108 Improved Retroviral vector design for gene therapy and regenerative medicine *Axel Schambach, Hannover Medical School; Boston Children's Hospital, Harvard Medical School*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

PROGRAMME

MONDAY 28 OCTOBER 2013

	INV109 Herpesviral vectors for the gene therapy of Friedreich's Ataxia *Javier Díaz-Nido, Universidad Autónoma de Madrid; CIBERER* *Proffered papers* OR071 Measles virus glycoprotein pseudotyped lentiviral vectors transduce resting long-term repopulation hCD34+ cells at an efficiency without precedent *Els Verhoeyen, CIRI EVIR team 69007, Lyon* OR072 Bromodomain and extra-terminal (BET) proteins target MLV-based vector integration to transcription start sites *Rik Gijsbers, Laboratory for Molecular Virology and Gene Therapy, KU Leuven, Belgium* OR073 Integration site analysis in a clinical trial of lentiviral vector based hematopoietic stem cell gene therapy for metachromatic leukodystrophy *Eugenio Montini, San Raffaele Telethon Institute for Gene Therapy, Milan*
11.00	Morning break
11.30-13.30	**Presidential symposium awards ceremony**
Auditorium	*Chairs: Luigi Naldini, Juan Bueren* **Young Investigator Awards** *SPP1230 Award "Mechanisms of Vector-Host Interactions"* OR074 Tracking individual hematopoietic stem cell activity *in vivo* in humans through integration site barcoding *Luca Biasco, San Raffaele Telethon Institute for Gene Therapy, Milan* OR075 AAV expression of CD39 and CD73 as a novel therapeutic strategy for the treatment of rheumatoid arthritis *Jonathan Finn, Arthrogen BV, Amsterdam* **Oustanding Achievement Award** INV110 The T-Body/CAR Approach: A long trip to the clinic with challenges yet to be met; the chronicle of an outstanding achievement *Zelig Eshhar, The Weizmann Institute of Science, Rehovot; Tel Aviv Sourasky Medical Center* **Presidential conference** INV111 Distinct bone marrow niches for distinct hematopoietic stem and progenitor cells *David Scadden, MGH Center for Regenerative Medicine , Harvard, Massachusetts*

NOTES

I never said it was possible. I only said it was true. —Charles Richet

A Gastronomic Sensation...

The Casino de Madrid was founded in 1836 when a group of young romantics and progressive liberals decided to search out a quiet place to meet in peace and harmony away from the frenetic world of politics. The significance of its artistic collection led the Casino de Madrid to be officially declared as a building of National Cultural Interest in 1993.

Casino de Madrid, Alcalá 15, 28014 Madrid (Metro Sevilla)

at the Casino de Madrid

Sunday 27 October 2013, 21:00
€20 per person

The Gala Evening will start with a standing dinner in the main reception room of the casino. The catering will be provided by Paco Roncero who is a 2 Michelin star chef. Roncero's scientific approach to the culinary arts comes from his years working under Ferran Adrià, the founder of gastronomic mecca "el Bulli". After this amazing culinary experience we will move to a different part of the Casino de Madrid for a fun disco night party where you will discover just what the word Fiesta really means!

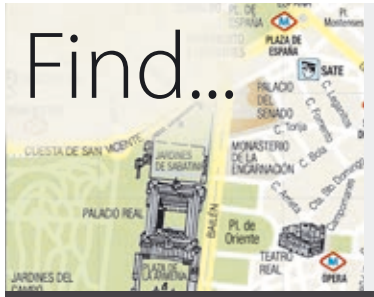
GALA DINNER IMAGES © WWW.PACORONCEROCATERING.COM

VISITING MADRID

Madrid is a bustling city, full of attractions that will delight and amaze any visitor. The capital and largest city of Spain, Madrid is located 646m above sea level and spans 698km^2. Below is a guide to help you find your way around the city and get the most out of your visit.

The Royal Palace, the Prado Museum, Almudena Cathedral... there is much to see and do in Madrid! Scan this code for tourist information and top ten attractions in Madrid.

www.madridtourist.info



Scan this code for an interactive tourist map of Madrid. You will find all the information you need to find your way around the city centre, plus phone numbers and addresses of top tourist attractions.

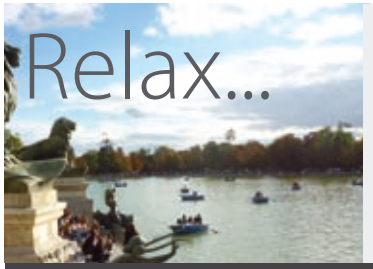
www.madridtourist.info/tourist_map.html

Madrid is known for its fish and seafood, and of course tapas. It is currently going through a gastronomical effervescence, which is sure to delight your taste buds! Scan this code for a guide on where to eat in Madrid.

www.to-madrid.com/madrid_guide/where_to_eat

For information on car parking see **www.madrid-tourist-guide.com/en/transport/car-parking-madrid.html**. However, the metro is the best way to get around Madrid. Scan this code for a downloadable map of the Metro.

http://www.metromadrid.es/en/viaja_en_metro/red_de_metro/planos/index.html

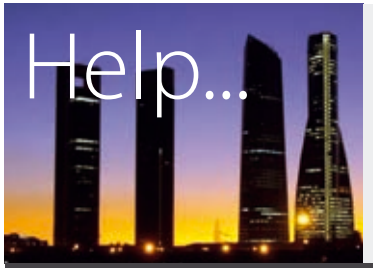


Madrid enjoys one of the most extensive areas of parks, gardens and "green zones" of all European cities. It possesses a total of 33 million square metres of parkland, distributed throughout more than 40 parks within the urban limits.

www.esmadrid.com/en/parks-madrid

Shopping in Madrid is varied and fascinating. Scan this code for a guide to where to shop, from the renowned Sunday fleamarket to the revamped Salamanca district.

www.esmadrid.com/en/shopping-madrid



Scan this code for more information on the practicalities of visiting Madrid, from where to find Tourist Information Centres, to Official Guided Tours, the Madrid Card and the Tourist Travel Pass.

www.esmadrid.com/en/tourist-information-madrid

EUROPEAN SOCIETY OF GENE AND CELL THERAPY ACHIEVEMENT AWARDS

Outstanding Achievement Award: In collaboration with Human Gene Therapy and Mary Ann Liebert Publishers, ESGCT presents one award for an established researcher who has made a long-term, outstanding contribution to the field. €2000 honorarium and 30 minute presentation during the annual congress.

Young Investigator Awards: €1000 and a 15 minute presentation during the annual congress for up to four researchers who are showing exceptional promise.

Travel grants: up to 10 awards of €250 for PhD and first post docs. These will be awarded on the basis of abstract score.

Application and nomination details are available at **www.esgct.eu/awards**

Note: Eligibility criteria applies

ESGCT EVALUATION

We do hope you have enjoyed the ESGCT/SETGyC Collaborative Congress 2013. We really value your feedback about all aspects of the Congress. We would be very grateful if you could take a few minutes to complete this online questionnaire either during or soon after the Congress.

https://www.surveymonkey.com/s/XPBDLXX

A link is also available from the ESGCT website.

If you enjoyed this meeting, would you consider hosting/organising an ESGCT or SETGyC congress in 2016? If so, please forward your proposal to the ESGCT Board as soon as possible at **office@esgct.eu**, or the SETGyC Board at **office@setgyc.es**

Thank you in advance for your time.

ESGCT and SETGyC Team

EuroFancoLen

Phase I/II Gene therapy trial of Fanconi anemia patients with a new Orphan Drug consisting of a lentiviral vector carrying the FANCA gene: A Coordinated International Action

Fanconi Anemia (FA) is a rare inherited syndrome characterized by the early development of bone marrow failure and increasing predisposition to cancer with age.

Allogeneic hematopoietic cell transplantation (alloHCT) is the only curative therapy for hematopoietic manifestations of FA, although associated with complications arising from myeloablation, graft versus host disease and increased incidence of squamous cell carcinoma.

The main objective of EuroFancoLen project is, therefore, the development of a multicentric Phase I/II gene therapy trial for FA-A patients, based on the genetic correction of plerixafor+G-CSF mobilized HSCs with the novel lentiviral vector, accompanied by comprehensive and groundbreaking safety and efficacy patient monitoring studies.

For further information, please contact us

Scientific Coordinator
Juan A. Bueren
juan.bueren@ciemat.es

Administrative Coordinator
Mario Romero
marioromero@idetra.com

eurofancolen.eu

European Union support to gene and cell therapy research (2007–2013)

Over the years the European Union, through its Framework Programmes for Research and Technological Development, has played an important role in supporting collaborative efforts in gene and cell therapy research, to develop tools, models and methods with the ultimate goal of bringing technology, mostly for treatment of debilitating, deadly or rare diseases, to the clinic and to the market.

In the field of gene transfer and gene therapy, more than €204 million have been invested since 2007. For stem cell research, the investments as of end December 2012 reached €647 million, including €185 million from ERC. In order to foster partnerships between multiple stakeholders and develop proof-of-concept, the last FP7 calls for gene and cell therapy required performance of clinical trials and involvement of small and medium-sized enterprises in projects. Recent news of new, often multicentre, clinical trials in gene therapy demonstrates the results of a policy that has put Europe in an international leading position. The next European programme for research and innovation Horizon 2020, for the period 2014-2020, will continue to support gene and cell therapy as innovative technologies for tackling health challenges.

David Gancberg

Directorate Health, Directorate-General for Research and Innovation, European Commission
CDMA 0/174, B-1049 Brussels, Belgium
Phone: +32 2 2984566
Fax: +32 2 2994693
E-mail: david.gancberg@ec.europa.eu

BRITISH SOCIETY FOR GENE AND CELL THERAPY – DATES FOR YOUR DIARY

British Society for Gene Therapy

PUBLIC ENGAGEMENT DAY

Friday 7th March 2014

Natural History Museum, Oxford

In collaboration with:
Oxfordshire Science Festival,
Genetic Disorders UK and hosted by the
University of Oxford Natural History Museum

GENE AND CELL THERAPY FOR INHERITED METABOLIC DISEASE MEETING

Thursday 27th March 2014

ICH, UCL, London

ANNUAL CONFERENCE

Friday 28th March 2014

ICH, UCL, London

PUBLIC ENGAGEMENT DAY

Tuesday 9th June 2015

Glasgow Science Centre, Scotland

GENE AND CELL THERAPY FOR INHERITED METABOLIC DISEASE MEETING

Tuesday 9th June 2015

Glasgow Science Centre, Scotland

ANNUAL CONFERENCE

Tuesday 9th – Thursday 11th June 2015

Glasgow Science Centre, Scotland

www.bsgct.org

DG-GT e.V.
Deutsche Gesellschaft für Gentherapie e.V.

ulm university universität ulm

German Society for Gene Therapy (DG-GT)

Barriers and Progress in Cell and Gene Therapy

Keynote
Luigi Naldini, Milano

Clinical Progress
Andrew Baker, Glasgow
Roger J. Hajjar, New York

Targeting the Genome
Zoltan Ivics, Langen
Angelo Lombardo, Milano

Next Generation Vector Technologies
Len Seymour, Oxford
Ernst Wagner, Munich
Hildegard Büning, Cologne

Using Cells as Therapeutic Vehicles
Dolores Schendel, Munich
Hans-Peter Kiem, Seattle

Vectored Anti-Tumor Strategies
Richard G. Vile, Rochester

Small and Non-Coding
Gunther Hartmann, Bonn

Regeneration
Marius Wernig, Stanford
Hartmut Geiger, Ulm

Opening Symposium: Production of Gene and Cell Therapeutics

XX Annual Meeting Ulm, 20-22 March 2014

University Medical Center
Albert-Einstein-Allee 23 · 89081 Ulm
Germany

Scientific Organisation:
Prof. Dr. Stefan Kochanek
Ulm University
Tel. +49 (0)731 500-46103
Fax +49 (0)731 500-46102
stefan.kochanek@uni-ulm.de
www.uni-ulm.de/dggt2014

FOR FURTHER INFORMATION VISIT WWW.UNI-ULM.DE/DGGT2014

Cancéropôle Grand Sud-Ouest

Bordeaux · Limoges · Montpellier · Nîmes · Toulouse

Toulouse

RÉGION MIDI-PYRÉNÉES

Société Française de Thérapie Cellulaire et Génique

FRENCH SOCIETY OF CELL AND GENE THERAPY

XII ANNUAL CONFERENCE

24 – 26 March 2014

Hôtel de Région, Toulouse, France

Satellite session, *Cancéropôle GSO*:

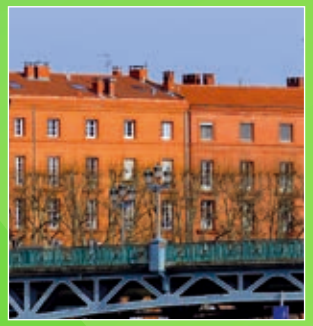
Cell and gene therapies, proof of concept to clinical trial

26 March 2014, 14:00

Hôtel de Région, Toulouse, France

---

www.sftcg.fr



NVGCT Nederlandse Vereniging voor Gen- & Celtherapie / Netherlands Society for Gene & Cell Therapy

NVGCT Spring Symposium March 13 & 14, 2014

Traditional program items

- Update of ZonMw projects: translational gene and cell therapy projects
- Greiner Award winner: best Ph.D. thesis of 2013
- Patient perspectives: invited lecture

Scheduled topics

- Stem cells & stem cell therapies
- Cancer gene therapy
- Inherited / neurodegenerative disorders
- Genetic vaccines
- Emerging technologies

Venue

Congrescentrum De Werelt, Lunteren www.congrescentrum.com

Updates of the program will become available at **www.nvgct.nl**

The Premier Journal on Gene Therapy

Celebrating 25 years in 2014!

Editor-in-Chief:
James M. Wilson, MD, PhD

Methods Editor:
Thierry VandenDriessche, PhD

Clinical Development Editor:
Barry J. Byrne, MD, PhD

The Official Journal of

ESGCT

SUOMEN GEENITERAPIASEURA
FINNISH SOCIETY OF GENE THERAPY

Sociedad Española Terapia Génica y Celular

NVGCT

British Society for Gene Therapy

Société Française de Thérapie Cellulaire et Génique

ISGCT The Irish Society for Gene & Cell Therapy

ISGCT איתג"ת

DG-GT e.V. Deutsche Gesellschaft für Gentherapie e.V.

Mary Ann Liebert, Inc. publishers

www.liebertpub.com/hgt

GENETHON
CURE THROUGH INNOVATION
PRIX GALIEN 2012

GENE THERAPY FOR RARE DISEASES

Advancing Therapies From Research to Patient Treatment

Created in 1990 by AFM-TELETHON and funded through the donations collected annually during the Telethon fundraising event, Genethon, a pioneer in deciphering the human genome and identifying the genes associated with genetic diseases is today fully dedicated to the **design, development and production of gene therapy treatments for rare diseases**.

In 2013, Genethon has opened Genethon BioProd, a 5000 m² facility fully dedicated to GMP-production and QC of gene therapy products.

FOR MORE INFORMATION and details:
www.genethon.fr

Member of BIOTHERAPIES INSTITUTE
CURE THROUGH INNOVATION

CASINO MADRID
(GALA DINNER)
POSADA DE LA VILLA
(SPEAKER DINNER)
A
B
C
D
E
F
G
H
01
02
03
04
05
06
07
08
09
10
PALACIO DE LIRIA
C. CULTURAL CONDE DUQUE
VENTURA RODRÍGUEZ
TEMPLO DE DEBOD
TORRE DE MADRID
EDIFICIO ESPAÑA
MUSEO CERRALBO
PL. DE ESPAÑA
PLAZA DE ESPAÑA
MONUMENTO A CERVANTES
Pl. Mostenses
NOVICIADO
TRIBUNAL
MUSEO DE HISTORIA DE MADRID
PRÍNCIPE PÍO
SATE
PALACIO DEL SENADO
SANTO DOMINGO
GRAN VÍA
CALLAO
CUESTA DE SAN VICENTE
JARDINES DE SABATINI
MONASTERIO DE LA ENCARNACIÓN
MONASTERIO DESCALZAS REALES
Pl. S. Martín
PALACIO REAL
Pl. de Oriente
TEATRO REAL
ÓPERA
PLAZA DE LA ARMERÍA
JARDINES DEL CAMPO DEL MORO
CATEDRAL DE LA ALMUDENA
BAILÉN
CALLE BAILÉN
PUERTA DEL SOL
SOL
REAL ACADEMIA DE BELLAS ARTES
SEVILLA
Pl. del Carmen
Pl. de Santiago
Pl. de S. Miguel
PLAZA MAYOR
CASA DE LA VILLA
PLAZA DE LA VILLA
I. STA. CRUZ
PALACIO STA. CRUZ
Pl. de Sta. Ana
TEATRO ESPAÑOL
Pl. del Ángel
PARQUE ATENAS
CALLE SEGOVIA
Pl. de la Paja
PARQUE DE LAS VISTILLAS
I.DE SAN ANDRÉS
I. DE SAN PEDRO
MUSEO ORÍGENES
COLEGIATA DE S. ISIDRO
TIRSO DE MOLINA
ANTÓN MARTÍN
FILMOTECA NACIONAL
Pl. Puerta de Moros
LA LATINA
PARQUE DE LA CORNISA
SAN FRANCISCO EL GRANDE
EL RASTRO
I. SAN CAYETANO
Pl. de Cascorro
GRAN VÍA DE S. FRANCISCO
RONDA DE SEGOVIA
LAVAPIÉS
Pl. de la Corrala
TEATRO VALLE-INCLÁN
PUERTA DE TOLEDO
Glorieta Puerta de Toledo
MERCADO PUERTA DE TOLEDO
PARQUE DEL RASTRO
RONDA DE TOLEDO
EMBAJADORES
CASA ENCENDIDA

LEYENDA

- METRO
- HOSPITAL
- CERCANÍAS
- INFORMACIÓN TURÍSTICA
- COMISARÍA (SATE)

INFORMACIÓN TURÍSTICA

OFICINAS DE TURISMO DEL AYUNTAMIENTO DE MADRID
(+ 34) 91 588 16 36
Lun - Dom de 09:30 a 20:30 h.
turismo@munimadrid.es
www.esMADRID.com

CENTRO DE TURISMO DE MADRID F-06
Plaza Mayor, 27
SOL / ÓPERA

CENTRO DE TURISMO COLÓN K-02
Plaza de Colón (paso subterráneo entre la calle Goya y la calle Génova)
COLÓN

PLAZA DE CIBELES K-05
Bulevar, esquina con el Paseo del Prado. Frente a parada de autobuses
BANCO DE ESPAÑA

PLAZA DE CALLAO F-04
Plaza de Callao, esquina con C/ Preciados
CALLAO

PASEO DEL ARTE K-09
Glorieta del Emperador Carlos V (Atocha) esquina con C/ Santa Isabel
ATOCHA

AEROPUERTO MADRID BARAJAS TERMINAL 4
Llegadas salas 10 y 11
AEROPUERTO T4

SERVICIO DE ATENCIÓN AL TURISTA EXTRANJERO (SATE) D-03
C/ Leganitos,19 (Comisaría de Centro)
SANTO DOMINGO
Plaza de España / Callao
De 09:00 a 22:00 h.
DENUNCIAS 24 HORAS: 902 102 112
satemadrid@munimadrid.es

¡MADRID!

01 02 03 04 05 06 07 08 09 10

I J K L M N Ñ

1944 Avery, MacLeod & McCarty **1953** Watson & Crick **1973** Graham & van der Eb **1989** Capecchi **1991** Culver, Anderson & Blaese **2000** Venter – Collins **2000** Cavazzana-Calvo et al **2012** EC approves first gene therapy

What's next...

The XXIInd Annual ESGCT Congress in collaboration with the NVGCT

23–26 October 2014

WorldForum, The Hague, The Netherlands

www.esgct.eu • www.nvgct.nl