ISSCR INTERNATIONAL SOCIETY FOR STEM CELL RESEARCH

INTERNATIONAL 2016 SYMPOSIA

EUROPEAN SOCIETY OF GENE & CELL THERAPY

XXIV[TH] ANNUAL CONGRESS

IN COLLABORATION WITH

abcd Associazione di Biologia Cellulare e del Differenziamento

CHANGING THE FACE OF MODERN MEDICINE:

STEM CELLS & GENE THERAPY

FLORENCE ITALY

18-21 OCTOBER 2016

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Welcome to...

FLORENCE

Palazzo dei Congressi e Palazzo degli Affari
Piazza Adua 1, 50123 Firenze

The Palazzo dei Congressi is hosted inside the nineteenth-century Villa Vittoria, named after the wife of Count Alessandro Contini Bonacossi, who purchased the villa in 1931.

In 1964, the villa was acquired by the Independent Tourist Board and turned into an International Congress Centre. It is surrounded by a century-old park and lies adjacent to the Limonaia (Lemon-House) and the Palazzo degli Affari.

Tel: +39 55 49721 • Email: info@firenzefiera.it
www.firenzefiera.it

Speaker ready room: Vasari

Press room: Filippo Lippi

Registration and Information Desk

For payment and membership queries and any other information regarding the Congress:

Tuesday 18 October 08.00–20.00
Wednesday 19 October 07.30–20.00
Thursday 20 October 07.30–20.00
Friday 21 October 08.30–17.00

Tourist Information Desk

Located next to Registration Desk.
Tuesday 18 October 14.00–19.00
Wednesday 19 October 08.00–18.00
Thursday 20 October 07.30–18.00
Friday 21 October 08.00–17.00

Information boards

Delegates may post CVs, employment opportunities or information on the designated boards located near the registration desk.

Abstracts

Electronic copies can be found in your registration profile. If you ordered a physical copy this can be collected from the registration desk.

In case of emergency, contact:

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

Useful Numbers

Florence Airport: +39 055 3061300

Florence Airport Lost Luggage: +39 055 3061302

Trenitalia (National Railway Info): +39 892021

Tourist Information Center in Via Cavour 1/r: +39 055 290832

Tourist Information Center in Piazza Stazione, 4: +39 055 212245

Taxi: +39 055 4242 / +39 055 4390

Emergency Numbers

Carabinieri (local police): 112

Police Emergency: 113

Fire Station: 115

Ambulance / First Aid: 118

Speaker hotel information:

Grand Hotel Baglioni
Piazza dell'Unità Italiana, 6

Tel: +39 055 23580
Fax: +39 055 23588895
Email: info@hotelbaglioni.it
www.hotelbaglioni.it

For more information about visiting Florence see page 94 or **www.firenzeturismo.it/en**. See inside back cover for map of Florence.

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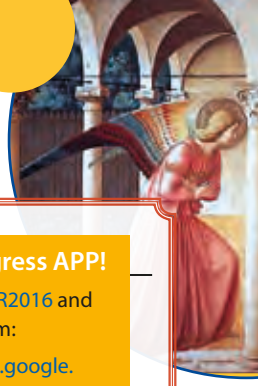

CONTENTS

Download the congress APP!

The name is ESGCT-ISSCR2016 and you can download it from:

google play: https://play.google.com/store/apps/details?id=com.gogoagenda.main.ESGCT&hl=en

or

iTunes: https://itunes.apple.com/it/app/esgct-isscr2016/id1137256537?mt=8

Or simply scan the QR code below!

Scanned with iOs devices it will link to iTunes, scanned with Android devices it will link to Google Play.

Organisers:

WATS.ON Ltd in collaboration with ISSCR:

Renée Watson
Gaëlle Jamar
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Sarah Pentecost

www.wats-on.co.uk
www.isscr.org

With special thanks to

AFFILIATED JOURNAL

ESGCT EXCELLENCE AWARDS

We are delighted to present an exceptional field of award winners in 2016

Outstanding Achievement

Amit Nathwani, *University College London*

IN093 Progress for gene therapy in haemophilia

Young Investigator

Pietro Genovese, *SR-Tiget, Milan*

OR063 Towards clinical translation of gene editing technologies for empowering adoptive immunotherapy or correcting inherited mutations

Travel Grants

Alessio Cantore, *SR-Tiget, Milan*

Pietro Giuseppe Mazzara, *San Raffaele Scientific Institute, Milan*

Margherita Norelli, *San Raffaele University, Milan*

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EUROPEAN SOCIETY OF GENE & CELL THERAPY

Adele Mucci, *RG Reprogramming and Gene Therapy, Cluster of Excellence REBIRTH, Hannover*

Miriam Hetzel, *Institute of Experimental Hematology, REBIRTH Cluster of Excellence, Hannover Medical School, Hannover*

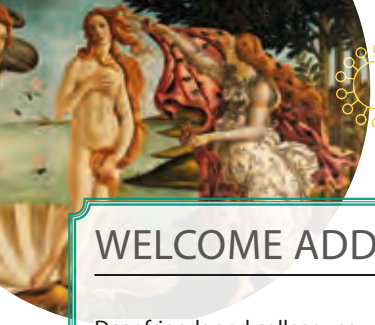
DG-GT e.V.

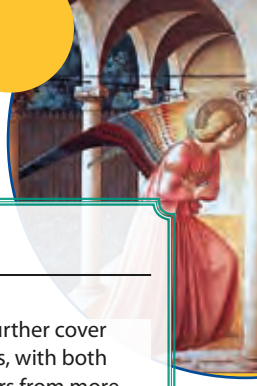
Nerea Zabaleta, *CIMA, University of Navarra, Pamplona*

Giulia Carola, *IBUB, University of Barcelona*

Fanny Collaud, *Genethon, Evry*

Saliha Majdoul, *Genethon, Evry*





WELCOME ADDRESS

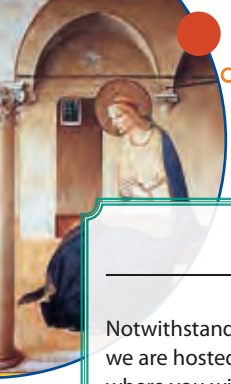
Dear friends and colleagues,

Welcome to Florence and to the first joint meeting of the European Society for Gene and Cell Therapy (ESGCT) and the International Society for Stem Cell Research (ISSCR), organised in collaboration with the Italian Association of Biologists working on Cells and Differentiation (ABCD).

We are here to celebrate exciting advances in our understanding of stem cell regulation, tissue development, regeneration, disease, and immune controls that, together with emerging powerful technologies of genetic engineering, are driving the design of novel cell and gene therapy approaches. Furthermore, progress on the clinical front continues to prove the potential of these strategies to deliver remarkable benefits to patients: more clinical trials are opening, the number and follow-up of participants is increasing, and the first cell and gene therapy products have now reached the market.

We will discuss these and many other findings in a first-rate scientific programme featuring:

- Keynote lectures by Hans Clevers and George Q Daley.
- 7 plenary sessions covering: neural disease modelling and neural stem cell transplantation; the biology and clinical applications of hematopoietic stem cells and skeletal and cardiac muscle stem cells; organoids; cancer immuno–gene therapy; new technologies for genome and epigenome editing; gene therapy in the market; and *in vivo* gene therapy.
- 21 parallel sessions which further cover these and many other topics, with both invited and selected speakers from more than 500 abstracts submitted to the meeting.
- 2 poster sessions, each offered for a full day.
- Education sessions leveraging the expertise of top scientists from our societies.
- 3 workshops discussing the challenges of translating the investigational new cell and gene therapies into clinical trials and eventually delivering them as commercially available drugs.
- A debate at the close of the meeting, addressing the scientific merit, technical challenges, and ethical aspects of editing the human germline.
- The presentation of the ESGCT 2016 Outstanding Achievement Award to Amit Nathwani and of the ESGCT 2016 Young Investigator Award to Pietro Genovese, in the ESGCT Presidential Session.
- A special joint issue of the journals Human Gene Therapy and Stem Cells and Development, available to all participants, offering short reviews and perspectives from many of our distinguished speakers on emerging scientific topics and opportunities for our field, new challenges ahead, and their thoughts on how to address them.
- A public forum before the start of the meeting allowing our scientists to speak to the local community and explain these advances and their meaning to patients and the community, organised together with the Telethon Foundation.



Notwithstanding the promise of great science, we are hosted in beautiful, historic Florence, where you will find an abundance of art masterpieces along the narrow streets and stunning squares (dating from the Middle Ages), or in each of the many churches and museums. More than anywhere else, this city celebrates the vision of Renaissance humanists, artists, engineers, and scientists who put human genius and its achievements, free thinking, and open-minded investigation at the centre of our intellectual and spiritual universe. I cannot resist citing the words that our most famous poet, the Florentine Dante Alighieri, put in Ulysses' mouth: "Fatti non foste a viver come bruti ma per seguir virtute e canoscenza", when he convinces his fellows to sail towards the unchartered seas, past the boundary of the known world, for the pursuit of virtue and knowledge, dismissing the calls and lures of material life. We are sure that this setting, besides pleasing our senses, will be an inspiration to our work, as we aim to lead transformative new scientific understanding and powerful technologies towards advancing our knowledge, and benefiting mankind by providing new treatment for diseases.

And if the science, arts, and humanism were not sufficient, you can also enjoy the unique Molecular Mingle social evening at the Mercato Centrale, a typical Italian market set in an original Art Nouveau building in one of the oldest squares of Florence. The market will be reserved for us and we will be able to sample fresh produce and high-quality food offered by local food artisans, followed by live music and dancing.

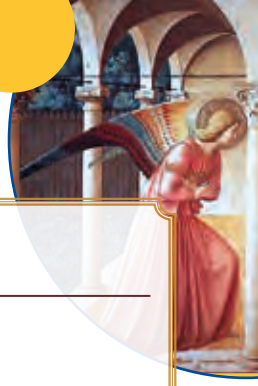
None of this would have been possible without the efforts and contribution of:

- the staff of our societies, who have worked unremittingly for the past year to organise the Congress and are here to help it run smoothly;
- the sponsors listed in the accompanying pages that have provided generous financial support;
- the members of the organising scientific committee, who have put together this remarkable programme;
- the invited speakers, many of whom have travelled from far away to be here and report their results;
- all of you, for your active participation and contributions.

We look forward to meeting you here and working together to make this a truly memorable event.

Luigi Naldini, Chair of the Organising Committee, with
Nathalie Cartier, President of ESGCT,
Sally Temple, President of ISSCR
Giuseppe Testa, ABCD



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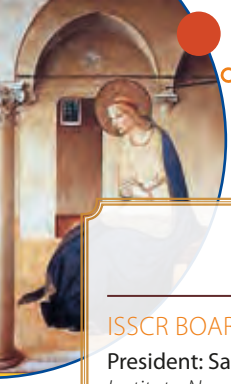
ISSCR INTERNATIONAL SOCIETY FOR STEM CELL RESEARCH



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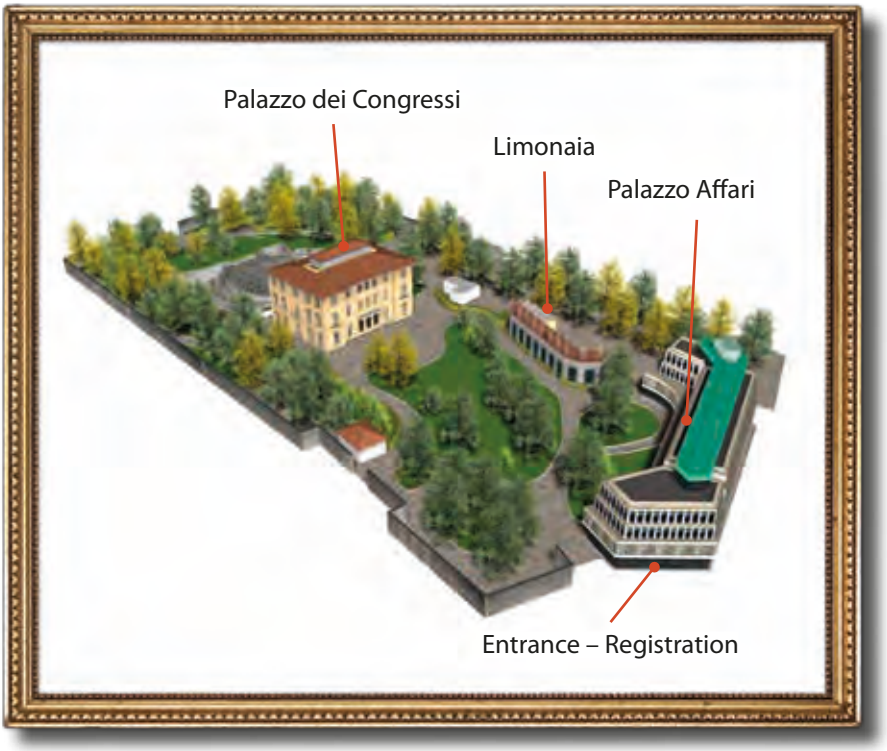
CONGRESS VENUE



PALAZZO AFFARI

Lower ground floor

Michelangelo (parallel sessions)

Ground floor

Botticelli (parallel sessions)

First floor

Piero Della Francesca (additional meeting room)

Masaccio (parallel sessions)

Second floor

Fra' Angelico (posters)

Leonardo (posters)

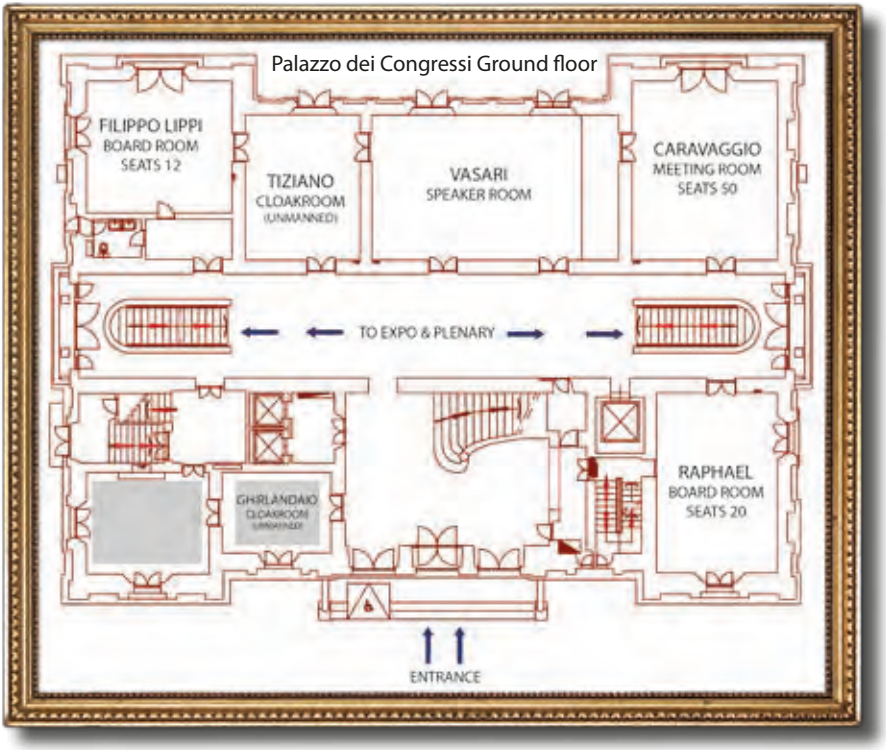
Donatello (additional meeting room)

Fourth floor

Paolo Uccello, Giotto (additional meeting rooms and posters)

LIMONAIA

Exhibition and catering



PALAZZO DEI CONGRESSI

-1

Brunelleschi Auditorium (plenary room)
Passi Perduti (exhibition hall and catering)
Ballatoi (posters)

Lower Ground

Hugo Club and Salone (exhibition hall and catering)

Ground floor

Vasari (speaker room)
Tiziano (cloakroom and left luggage)
Filippo Lippi (press room)
Caravaggio, Raffaello (additional meeting rooms)

VISIT
STANDS 1 AND 2
FOR MORE INFORMATION

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Strimvelis gene therapy for ADA-SCID

Strimvelis is indicated for the treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID), for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available (see SPC for more information).[1]

The first licensed autologous *ex vivo* gene therapy in the EU

Email **contact.adascid@gsk.com** to find out more.

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Strimvelis®▼
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Strimvelis is not marketed in all EU countries.

Submitted to AIFA 03/10/2016 (Ufficio informazione medico scientifica ai sensi degli art. 119-120 del D.L.vo n. 219/06).

Reference
1. Strimvelis Summary of Product Characteristics. RD/SVE/0049/16 Date of preparation: September 2016

PRESCRIBING INFORMATION
Please consult the Summary of Product Characteristics before prescribing. **STRIMVELIS®** ▼ (autologous CD34+ cells transduced to express ADA). Finished product composed of one or more ethylene vinyl acetate (EVA) bags containing an autologous CD34+ enriched cell fraction transduced with retroviral vector to encode for the human ADA cDNA sequence. Quantitative information is presented in labelling for each batch; concentration is 1-10 million CD34+ cells/ml. **Indication**: Treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID) for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available. **Dosage and administration**: Must be administered in specialist transplant centre by physician with significant previous experience of managing ADA-SCID patients and use of autologous CD34+ *ex vivo* gene therapy products. Should only be administered after consultation with patient and/or family. Patient must be able to donate adequate CD34+ cells to deliver the minimum 4 million purified CD34+ cells/kg required for manufacture of Strimvelis. Strimvelis is for autologous use only. Recommended dose range is between 2 and 20 million CD34+ cells/kg. Should be administered once only via intravenous (IV) infusion. Infusion rate should not exceed 5ml/kg/hr; infusion period is approximately 20 minutes. **Pre-treatment conditioning**: Recommend that 0.5mg/kg IV busulfan administered every 6 hours on two consecutive days starting three days before Strimvelis administration (total busulfan dose 4mg/kg, divided into 8 doses of 0.5mg/kg). Plasma busulfan levels must be measured after first dose each day. If AUC exceeds 4000ng/ml*h, dosage should be reduced as required. **Pre-medication**: Recommend IV antihistamine administered 15-30 minutes before Strimvelis infusion. *Children:* Safety and efficacy in children <6 months of age or >6 years and 1 month has not been established; no data available. *Elderly:* Not intended for use in patients >65 years of age. *Hepatic and renal impairment:* Not studied; no dose adjustment expected to be required. **Contraindications**: History of hypersensitivity to the product or excipients (sodium chloride), current or previous history of leukaemia or myelodysplasia, positive HIV test or test for any other agent listed in the current EU Cell and Tissue Directive, and history of previous gene therapy. **Special warnings and precautions**: Should never be administered to any patient other than the original CD34+ cell donor. **Use with caution** in patients <6 months of age and >6 years and 1 month and with hypersensitivity to aminoglycosides or bovine serum albumin. There is a **potential risk** of leukaemic transformation following treatment with Strimvelis. Recommend patients are monitored long-term. **Long-term effects and durability of response to Strimvelis on ADA-SCID are unknown**. Patients should be closely monitored for occurrence of severe and opportunistic infections, immune reconstitution parameters and need for replacement IV immunoglobulin (IVIG). **There have been cases where treatment with Strimvelis has been unsuccessful**. Some patients have had to resume long-term enzyme replacement therapy and/or receive a stem cell transplant. Non-immunological manifestations of ADA-SCID may not respond. No immunogenicity testing has been conducted. Patients can develop autoimmunity. **Patients treated with Strimvelis should not donate blood, organs, tissues and cells for transplantation at any time in the future**. **Interactions**: No interaction studies have been performed. **Fertility, pregnancy and breastfeeding**: *Women of child-bearing potential:* As Strimvelis will be administered following busulfan conditioning patients of child-bearing potential must use reliable barrier contraception during administration and for at least 6 months after. *Pregnancy:* No clinical data on exposed pregnancies; reproductive and developmental toxicity studies were not performed. Should not be used during pregnancy. *Breastfeeding:* Should not be administered to women who are breastfeeding. *Fertility:* No data available. **Side effects**: Safety evaluated in 18 subjects with median duration of follow-up of 7 years. *Very common (≥1/10):* Anaemia, neutropenia, hypothyroidism, hypertension, asthma, allergic rhinitis, atopic dermatitis, eczema, pyrexia, hepatic enzyme increased, antinuclear antibody (ANA) positive. *Common (≥1/100 to <1/10):* Autoimmune haemolytic anaemia, autoimmune aplastic anaemia, autoimmune thrombocytopenia, autoimmune thyroiditis, Guillain-Barré syndrome, autoimmune hepatitis, anti-neutrophil cytoplasmic antibody positive, smooth muscle antibody positive. *Serious:* Autoimmunity (e.g. autoimmune haemolytic anaemia, autoimmune aplastic anaemia, autoimmune hepatitis, autoimmune thrombocytopenia and Guillain-Barré syndrome). All side effects are considered to be related to immune reconstitution (due to their nature and timing) or potentially related to busulfan. **Prescribers should consult the summary of product characteristics for complete information regarding the adverse reaction profile. Legal category**: POM. **Marketing authorisation (MA) number**: EU/1/16/1097/001. **MA holder**: GlaxoSmithKline Trading Services Limited, Currabinny, Carrgaline, County Cork, Ireland. **Date of preparation**: August 2016. RD/SVE/0070/16

▼This medical product is subject to additional monitoring. Adverse events should be reported. Reporting forms and information can be found at http://www.mhra.gov.uk/yellowcard. Adverse events should also be reported to GlaxoSmithKline on 0800 221 441 if you are a UK physician.

Healthcare professionals practising outside the UK should report adverse events to their local GSK office and follow their national guidance on adverse event reporting.

GETTING SOCIAL WITH ESGCT AND ISSCR

Follow our official channels on social media:

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@ESGCT and @isscr

@ESGCT

Make sure you use the official hashtags **#ESGCT16 and #isscr** in your posts, and check out the latest Congress news and updates! Come and visit us at the ESGCT and ISSCR booths. You can find information on the next Spring School and the Berlin Congress 2017, as well as the ISSCR 2017 annual meeting in Boston. Join in at our social media hub, play games, and win prizes! Buy dinner tickets and pick up drinks vouchers for the dinner amongst all the other things. Or just pop by to say hi! Not to be missed!

Look out for our Congress Mascots! They'll be around and about, and will be busy posting photos and messages throughout the Congress. Find them and take a picture with them... you might win a prize!

Try the virus quiz! Have you ever wondered… if you were a virus, which virus you would be? Take the definitive quiz to find out at **www.uquiz.com/OLnLtv**

Are you ready to get social? We have a few challenges waiting for you!

We will award prizes to:

- The best Congress photo
- The best Florence photo
- The best Mascot photo
- The most retweeted Congress tweet
- The most liked Instagram photo

Only posts with the official hashtags **#ESGCT16** and **#isscr** will be considered! Bear in mind your privacy settings – if we can't see your posts, we can't include them in the contest.

Any questions? Come and speak to us at the ESGCT or ISSCR booths or tweet us **@ESGCT** and **@isscr** and we will be happy to help!

PROGRAMME AT A GLANCE

TUESDAY 18 OCTOBER 2016

CLINICAL TRIAL AND COMMERCIALISATION WORKSHOP *First floor: Piero della Francesca room*		P. 66
08.00-09.00	Registration	
09.00-09.20	Planning a clinical trial	
09.20-10.00	Manufacturing of gene and cell products	
	Coffee available in the room	
10.00-10.20	Gene and cell therapy technologies	
10.20-11.20	Pricing and reimbursement	
11.20-11.40	Academic vs commercial clinical development strategy	
11.40-12.00	Regulatory strategy in gene and cell therapy development	
12.00-12.40	Finding the value	
12.40-13.00	Elevator pitches	

EDUCATION DAY *Morning session: -1 floor Michelangelo* *Afternoon session: Brunelleschi Auditorium* *Sponsors: Supersist; Dimension Therapeutics*		P. 68
08.00-09.00	Registration	
09.00-09.30	E1: Opening words	
09.30-10.30	E2a: Tailoring gene transfer vectors	
10.30-11.00	E2b: Disease modelling	
11.00-11.30	Coffee Break	
11.30-12.30	E3: Stem cells and iPS – current state	
12.30-13.30	Lunch – *Limonaia, Passi Perduti*	
13.30-14.30	E4a: Immunotherapy and transdifferentiation	
14.30-15.30	E4b: Gene editing	
15.30-16.00	Coffee break	

PROGRAMME AT A GLANCE

TUESDAY 18 OCTOBER 2016

PUBLIC ENGAGEMENT DAY FOR PATIENT ASSOCIATIONS AND SCIENCE DIALOGUES WITH CITIZENS: **Leading edge therapies for rare diseases** *First floor, Masaccio room* *Sponsors: Telethon Foundation, Dimension Therapeutics, Supersist, AFM Telethon*		P. 70
09.00-09.45	Registration	
09.45-10.15	Gene therapy	
10.15-10.45	How to foster access to therapies	
10.45-11.15	Safety studies	
11.15-11.45	Science and bio-ethics	
11.45-13.00	Discussion	
13.00-14.00	Lunch and networking (in the room)	
14.00-14.30	New frontiers in science	
14.30-16.30	Role playing: science dialogues	
16.30-17.00	Closing remarks	

PROGRAMME AT A GLANCE

TUESDAY 18 OCTOBER 2016

MOLMED SYMPOSIUM **An entrepreneurial approach to translate academic knowledge into therapeutic solutions for all patients** *Ground floor, Botticelli room* *Sponsor: Molmed*		P. 71
13.00-14.00	Registration	
14.00-14.15	When pioneers in cell and gene therapy come up with a 'business' idea	
14.15-14.30	Academia ready to be a productive partner for biotech companies	
14.30-14.45	How the financial market operated and operates in sustaining the biotech sector development	
14.45-15.00	A picture of the European biotech sector: strengths and weaknesses	
15.00-15.30	Round table	

MAIN CONGRESS		
16.00-17.00	**ESGCT / ISSCR 2016 Opening: welcome and introduction and Opening Keynote** *Auditorium* *Sponsor: bluebird bio*	P. 73
17.00-19.00	**1: Neural diseases: modelling, reprogramming and transplantation in brain and retina** *Auditorium* *Sponsor: RegenXbio*	
19.00-20.00	Welcome reception *Limonaia, Passi Perduti*	
19.00-21.00	Molecular therapy 'meet the editor' reception *Ucello room* *Sponsor: Molecular Therapy*	P. 73

PROGRAMME AT A GLANCE

WEDNESDAY 19 OCTOBER 2016

MAIN CONGRESS					
08.30-10.30	**2: Hematopoietic stem cells: from biology to clinical applications** *Auditorium* *Sponsor: Genethon*				P. 74
10.30-11.00	Coffee break – *Limonaia, Passi Perduti*				
11.00-12.30	**2a: Imaging stem cells dynamics** *Botticelli*	**2b: Eye stem cell and gene therapy** *Masaccio* *Sponsor: MeiraGTX*	**2c: Central nervous system gene therapy** *Auditorium* *Sponsor: JSGT, Lysogene*		P. 74–75
12.30-14.00	Lunch – *Limonaia, Passi Perduti* *Posters available for viewing*				
12.45-13.45	**Lunch Symposium: Regulatory workshop for ATMPs** *Michelangelo* *Sponsor: BioReliance*				P. 75
14.00-16.00	**3: Skeletal and cardiac muscle stem cells: from biology and reprogramming to clinical applications** *Auditorium* *Sponsor: FinVector*				P. 76
16.00-16.30	Coffee break – *Limonaia, Passi Perduti*				
16.30-18.30	**3a: Organoids and high throughput platforms** *Botticelli*	**3b: Stem cell-based neural disease modelling** *Masaccio*	**3c: Cardiovascular gene and cell therapy** *Michelangelo* *Sponsor: CellforCure*	**3d: Immunology/ cancer immuno-gene therapy I** *Auditorium*	P. 76–78
18.30-20.30	Poster session 1 (Odd poster numbers). See page 42 for details				
20.00-23.00	Speaker dinner (by invitation only) *Palazzo Vecchio*				

PROGRAMME AT A GLANCE

THURSDAY 20 OCTOBER 2016

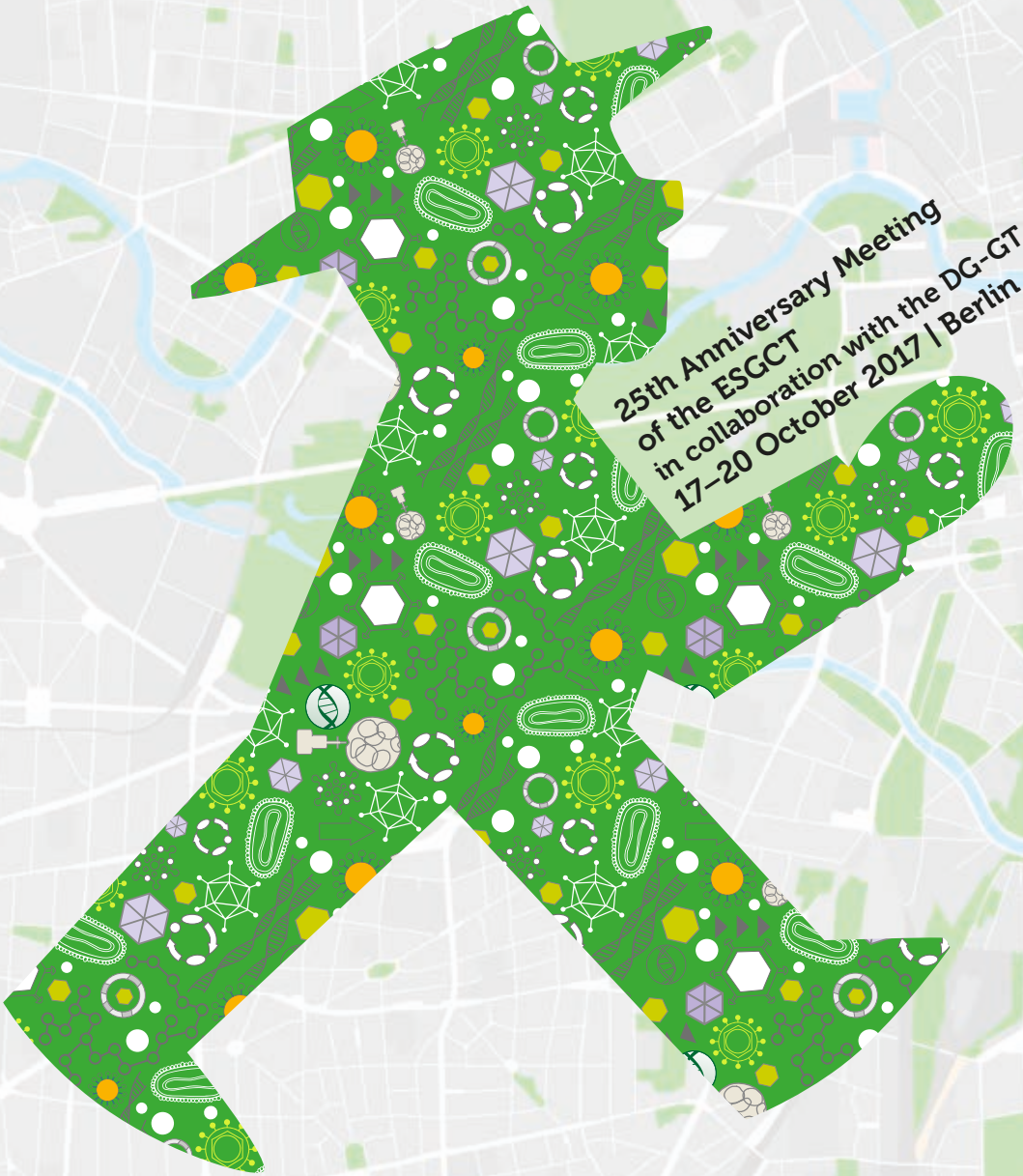
MAIN CONGRESS				
08.00-10.00	**4: Cancer immuno–gene therapy** *Auditorium* *Sponsors: Oxford BioMedica*			P. 80
10.00-10.30	Coffee break – *Limonaia, Passi Perduti*			
10.30-12.30	**4a: Haematopoietic stem cells and homeostasis** *Auditorium*	**4b: MSC gene and cell therapy** *Botticelli*	**4c: *In vivo* gene therapy I** *Masaccio* *Sponsors: Spark Therapeutics*	P. 80–81
12.30-14.00	Lunch – *Limonaia, Passi Perduti* *Posters available for viewing*			
14.00-16.00	**5a: Cancer stem cells** *Masaccio*	**5b: *Ex vivo* HSC-based gene and cell therapy** *Auditorium* *Sponsor: Molmed*	**5c: DNA-based gene transfer and *in vivo* gene therapy II** *Botticelli* *Sponsor: Adverum*	P. 82–83
16.00-16.30	Coffee break – *Limonaia, Passi Perduti*			
16.30-18.30	**5: New technologies: targeted genome and epigenome editing, new vector design, organoids** *Auditorium* *Sponsor: Editas Medicine*			P. 84
18.30-20.00	Poster session 2 (Even poster numbers) See page 42 for details			
20.30-01.00	Molecular Mingle evening – Mercato Centrale. See page 64			

PROGRAMME AT A GLANCE

FRIDAY 21 OCTOBER 2016

MAIN CONGRESS					
09.00-10.30	**6a: RNA-based gene transfer and integration studies** *Michelangelo*	**6b: Genome editing and gene correction** *Auditorium* *Sponsor: Intellia Therapeutics*	**6c: Cancer gene therapy** *Masaccio* *Sponsor: JSGT*	**6d: Immunology and allergy** *Botticelli*	P. 86–87
10.30-11.00	Coffee break – *Limonaia, Passi Perduti*				
11.00-12.00	**6: Gene therapy in the market** *Auditorium* *Sponsor: GlaxoSmithKline*				P. 87
12.00-13.00	**7: *In vivo* gene therapy** *Auditorium* *Sponsor: Biogen*				P. 88
13.00-14.00	Lunch – *Limonaia, Passi Perduti*				
14.00-15.30	**7a: Immunology/ cancer immuno-gene therapy II** *Auditorium*	**7b: Gene silencing: from small non-coding RNAs to epigenetic editing and gene disruption** *Botticelli* *Sponsor: Sangamo BioSciences*	**7c: Manufacturing of cell and gene therapy products** *Michelangelo* *Sponsor: Molmed*	**7d: CNS gene therapy** *Masaccio* *Sponsor: Avexis*	P. 88–89
15.30-15.50	Coffee break – *Limonaia, Passi Perduti*				
15.50-17.45	**ESGCT AGM and presidential symposium and awards ceremony** *Auditorium* *Sponsor: Bayer*				P. 90
17.45-19.00	**Germline editing debate** *Auditorium*				P. 90
19.00-20.00	Closing drinks				

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



Keynote speakers:

Chris Baum, Jef Boeke, Nathalie Cartier-Lacave

Plenary speakers include:

John Bell, Thomas Blankenstein, Malcolm Brenner, Frank Buchholz, Juan Bueren, Laurence Cooper, Michele de Luca, Stefanie Diemmeler, Giuliana Ferrari, Keith Joung, Juergen Knoblich, Andras Nagy, Adrian Thrasher

Parallel speakers include:

Eric Alton, Marinee Chuah, Giulio Cossu, Krithika Hariharan, Michael Hudecek, Eugenio Montini, Rosario Perona, Waseem Qasim, Axel Schambach, Len Seymour, Gabriele Thumann, Hans Dieter Volk, Christof von Kalle, David Williams, Guy Ungererchts

Plenary sessions on:

Highlight of clinical progress
Stem cells: biology, manipulation and reprogramming
Cancer immuno-gene therapy
New tools and technology: gene and genome editing and engineering
Gene and cell therapy in the market

Parallel sessions on:

iPS disease modelling
Ocular and central nervous system gene and cell therapy
Oncolysis
Gene editing
Cardiovascular, muscle and pulmonary gene and cell therapy
Vector development
Regenerative therapies
Metabolic and lysosomal storage diseases
Cancer predisposition, ageing and genetic instablility syndromes
Blood disorders
Cancer gene therapy

For updates and registration information see:
www.esgct.eu • www.dg-gt.de

Gene and Cell therapy support in the European Union: the Horizon 2020 research programme (2014–2020)

The 8th European Union (EU) programme for Research and Innovation, Horizon 2020 (2014–2020), supports the gene and cell therapy field by publishing calls for proposals for (clinical) collaborative research on chronic or rare diseases, in regenerative medicine, or for new technological developments, amongst other things.

Small and medium-size entreprises (SMEs) in the field can apply, even as a single partner, via a dedicated SME instrument. US partners are welcome throughout the health research programme and can be funded as well.

Other funding opportunities for researchers, such as the Marie Sklodowska-Curie actions (training), the European Research Council grants (individual frontier research), the Innovative Medicines Initiative projects (public-private partnership with the European Federation of the Pharmaceutical Industries and Associations), are also available on regular basis.

The first Horizon 2020 projects dealing with gene and/or cell therapy supported in 2014–2016 will be presented as well as the trends for the future Health programme.

Participant portal:
http://ec.europa.eu/research/participants/portal/desktop/en/opportunities/h2020

IMI: https://www.imi.europa.eu/

ERC: https://erc.europa.eu/

Contact: Dr David Gancberg
Directorate Health, Directorate-General for Research and Innovation, European Commission
CDMA 00/174, B-1049 Brussels, Belgium
Phone: +32 2 2984566 Fax: +32 2 2994693
Email: david.gancberg@ec.europa.eu

Advance your gene and cell therapy Manufacturing

- Integrated cell processing solutions
- Process innovation and development
- cGMP viral vector production
- Cell banking and analytical services

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PS-16-13186

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Based in Chicago, Illinois, AveXis is a clinical-stage gene therapy company dedicated to developing and commercialising novel treatments for patients suffering from rare and life-threatening neurological genetic diseases. Our initial product candidate, AVXS-101, is our proprietary gene therapy product candidate currently in a Phase 1 clinical trial for the treatment of spinal muscular atrophy (SMA) Type 1, the leading genetic cause of infant mortality. SMA Type 1 is a lethal genetic disorder characterised by motor neuron loss and associated muscle deterioration, resulting in mortality or the need for permanent ventilation support before the age of two for greater than 90% of patients. We are passionately committed to moving gene therapies into the clinical setting for patients and families devastated by rare and orphan neurological genetic diseases. With the support of industry and academic alliances, we're advancing cutting-edge science in order to treat rare and life-threatening genetic diseases – starting with our clinical-stage, proprietary gene therapy candidate, AVXS-101.

www.avexis.com

Editas Medicine is a transformative genome editing company founded by world leaders in the fields of genome editing, protein engineering, and molecular and structural biology, with specific expertise in CRISPR/Cas9 and TALENs technologies. The company's mission is to translate its genome editing technology into a novel class of human therapeutics that enable precise and corrective molecular modification to treat the underlying cause of a broad range of diseases at the genetic level. The company has generated substantial patent filings and has access to intellectual property covering foundational genome-editing technologies, as well as essential advancements and enablements that will uniquely allow the company to translate early findings into viable human therapeutic products.

www.editasmedicine.com

GlaxoSmithKline (GSK) has an established history of successfully researching and developing orphan drugs to treat rare diseases. Recognising the size of the challenge, but also the opportunity to deliver new medicines to patients, we announced the creation of a dedicated rare diseases unit in February 2010. Initially focusing on 200 rare diseases, we are collaborating with organisations and institutions to develop medicines, including gene and cell therapies, quicker and more effectively than ever before.

www.gsk.com

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MolMed S.p.A. is a medical biotechnology company focused on research, development and clinical validation of novel anti-cancer therapies. MolMed's pipeline includes anti-tumour therapeutics in clinical and preclinical development: Zalmoxis® (TK) is a cell therapy enabling bone marrow transplants from partially compatible donors without need of post-transplant immune-suppression, in Phase III in high-risk acute leukaemia and granted a Conditional Marketing Authorisation by the EC; NGR-hTNF is a novel therapeutic agent for solid tumours investigated in a broad clinical programme with more than 1,000 treated patients; CAR-CD44v6 is an immuno-gene therapy project with therapeutic potential for haematological malignancies and epithelial tumours, in preclinical development. MolMed also offers to third parties market-grade development and manufacturing services in cell and gene therapy. MolMed is listed on the main market (MTA) of the Milan stock exchange (ticker Reuters: MLMD.MI).

www.molmed.com

Oxford BioMedica (LSE:OXB) is a leading gene and cell therapy company focused on developing life-changing treatments for serious diseases. The company has built a sector leading lentiviral vector delivery platform (LentiVector®) through which it continues to develop *in vivo* and *ex vivo* gene and gene-modified therapies products both in-house and with partners. Oxford BioMedica has entered into a number of key partnerships, including with Novartis, Sanofi, GSK, Green Cross Lab Cell and Immune Design. Oxford BioMedica has world-class facilities and capabilities, encompassing the full range of GMP manufacturing and analytical activities to support preclinical, research and bioprocessing development through to GMP production and supply of clinical trial materials. The production activities are focussed on the manufacture of lentiviral vectors from human cell lines, both in adherent and large scale serum-free suspension culture. Oxford BioMedica is based across several locations in Oxfordshire, UK, and employs more than 230 people.

www.oxfordbiomedica.co.uk

DIAMOND PARTNERS

Spark Therapeutics, a fully integrated gene therapy company, is seeking to transform the lives of patients with debilitating genetic diseases by developing one-time, life-altering treatments. Spark Therapeutics' validated gene therapy platform is being applied to a range of clinical and preclinical programmes addressing serious genetic diseases, including inherited retinal diseases, liver-associated diseases such as haemophilia, and neurodegenerative diseases. Spark Therapeutics' validated platform has successfully delivered gene therapies with proof-of-concept data in the eye and liver. Spark Therapeutics' most advanced product candidate, voretigene neparvovec (formerly referred to as SPK-RPE65), which has received both breakthrough therapy and orphan product designations, reported positive top-line results from a pivotal Phase 3 clinical trial for the treatment of a rare blinding condition. Spark Therapeutics' haemophilia franchise has two lead assets: SPK-9001 in a Phase 1/2 trial for haemophilia B and SPK-8011, a preclinical candidate for haemophilia A. To learn more, please visit:

www.sparktx.com.

The goal of Supersist's project is the clinical translation of new gene targeting technologies for correcting inherited mutations and empowering adoptive immunotherapy of cancer. Substantial evidence supports the therapeutic potential of *ex vivo* gene therapy based on hematopoietic stem cell (HSC) or T lymphocytes to treat inherited diseases or cancer.

www.supersist-project.eu

ADVERUM
BIOTECHNOLOGIES

GAINING MOMENTUM IN GENE THERAPY

- Core capabilities in vector optimization, process development, assay development and manufacturing
- Robust pipeline focused on the patients
- Potential for long-term treatment benefits

Adverum is a gene therapy company committed to discovering and developing novel medicines that can offer life-changing benefits to patients who currently have limited or burdensome treatment options. Adverum is leveraging its next-generation adeno-associated virus (AAV)-based directed evolution platform to generate product candidates designed to provide durable efficacy by inducing sustained expression of a therapeutic protein.

PLATINUM PARTNERS

Adverum is a gene therapy company committed to discovering and developing novel medicines that can offer life-changing benefits to patients living with rare diseases or diseases of the eye who currently have limited or burdensome treatment options. Adverum has a robust pipeline and is leveraging its next-generation adeno-associated virus (AAV)-based directed evolution platform to generate product candidates designed to provide durable efficacy by inducing sustained expression of a therapeutic protein. Our focus on the patient is supported by clinical development expertise and core capabilities in vector optimisation, process development, manufacturing, and assay development.

www.adverum.com

Through cutting-edge science and medicine, Biogen discovers, develops, and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hematologic conditions and autoimmune disorders. Founded in 1978, Biogen is one of the world's oldest independent biotechnology companies and patients worldwide benefit from its leading multiple sclerosis and innovative haemophilia therapies.

www.biogen.com

Merck, through its brands BioReliance and SAFC, is a trusted manufacturer of specialty chemicals and biologics for commercial life science applications. We provide unique and innovative technologies and services for customers requiring a reliable partner throughout the development and manufacturing process. Merck offers world class process development, manufacturing and testing capabilities for virus-based therapeutic products. Visit us at our booth to discuss our clinical and commercial Virus and Gene Therapy Manufacturing services. Sigma-Aldrich Corp. is a subsidiary of Merck KGaA, Darmstadt, Germany.

www.bioreliance.com

With its lentiviral-based gene therapies, T cell immunotherapy expertise and gene editing capabilities, bluebird bio has built an integrated product platform with broad potential application to severe genetic diseases and cancer. bluebird bio's gene therapy clinical programmes include its Lenti-D™ product candidate for the treatment of cerebral adrenoleukodystrophy and its LentiGlobin™ BB305 product candidate for the treatment of transfusion-dependent ß-thalassemia and severe sickle cell disease. bluebird bio's oncology pipeline is built upon the company's leadership in lentiviral gene delivery and T cell engineering. bluebird bio's lead oncology programme,

PLATINUM PARTNERS

bb2121, is an anti-BCMA CAR T programme partnered with Celgene. bluebird bio also has discovery research programs utilising megaTALs/ homing endonuclease gene editing technologies with the potential for use across the company's pipeline.

www.bluebirdbio.com

Intellia Therapeutics is a leading gene editing company, focused on the development of proprietary, potentially curative therapeutics using the CRISPR/Cas9 system. Intellia believes the CRISPR/Cas9 technology has the potential to transform medicine by permanently editing disease-associated genes in the human body with a single treatment course. Our combination of deep scientific expertise and clinical development experience, along with our leading intellectual property portfolio, puts us in a unique position to unlock broad therapeutic applications of the CRISPR/Cas9 technology and create a new class of therapeutic products. Intellia was named as one of the top 10 biotech start-ups by Nature Biotechnology. In September 2015, Intellia was named a "Fierce 15" biotech company by FierceBiotech.

www.intelliatx.com

MeiraGTx is focused on the development of novel gene therapies for inherited and acquired disorders. The company is developing therapies for ocular diseases, including rare inherited blindness and wet and dry AMD, xerostomia following radiation treatment for head and neck cancer, and neurodegenerative diseases such as amyotrophic lateral sclerosis (ALS). MeiraGTx also has an innovative gene regulation platform that provides the potential to expand the way gene therapy can be applied to create a new paradigm for biologic therapeutics.

www.meiragtx.com

REGENXBIO is a leading biotechnology company focused on the development, commercialisation and licensing of recombinant adeno-associated virus (AAV) gene therapy. Our NAV® Technology Platform, a proprietary AAV gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercialising *in vivo* gene therapy products based on our NAV Technology Platform. We seek to accomplish this mission through a combination of our internal development efforts and the efforts of our third-party licensees.

www.regenxbio.com

GOLD PARTNERS

Bayer: Science For A Better Life. Bayer is a global enterprise with core competencies in the Life Science fields of health care and agriculture. Its products and services are designed to benefit people and improve their quality of life.

www.bayer.com

CELLforCURE is a pharmaceutical cell and gene therapy CDMO (Contract Development and Manufacturing Organisation) with strong knowledge and experience in cell and gene manufacturing. CELLforCURE proposes a one stop shop service from bench to patient and market, including:

- optimisation and industrialisation of processes;
- GMP/GMP manufacturing of clinical and commercial batches of cell and gene therapy products; and
- regulatory services and pharmaceutical distribution.

www.cellforcure.com

Dimension Therapeutics, Inc. (NASDAQ: DMTX) is the leader in discovering and developing new therapeutic products for people living with devastating rare diseases associated with the liver, based on the most advanced, mammalian adeno-associated virus (AAV) gene delivery technology. Dimension is actively progressing its broad pipeline, which features programmes addressing unmet needs for patients suffering from inherited metabolic diseases, including OTC deficiency, GSDIa, citrullinemia type 1, PKU, Wilson disease, a collaboration with Bayer in haemophilia A, and a wholly owned clinical programme in haemophilia B. The company targets diseases with readily identifiable patient populations, highly predictive preclinical models, and well-described, and often clinically validated, biomarkers. Founded in 2013, Dimension maintains headquarters in Cambridge, Massachusetts.

www.dimensiontx.com

FinVector is a world leader in the research and development of viral-based gene therapy products, with state-of-the-art facilities and a highly experienced scientific team working in the gene therapy market. We deliver a tailored service to meet and exceed our clients' needs, and use our scientific expertise and industry knowledge to help clients take viral-based products from the preclinical phase, through clinical trials and to market. Come and visit us at booth 28–29.

www.finvector.com

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Genethon, created by AFM Téléthon, has the mission to make innovative gene therapy treatments available to patients affected by rare genetic diseases. To meet this challenge, Genethon has assembled the technical and human resources needed to accelerate the medical application of scientific discoveries arising from fundamental research. Strong translational research programmes engage multi-disciplinary teams and are supported by a first-rate technological platform and cGMP facility. The pipeline of Genethon includes products currently in international clinical trials and at preclinical stages, for muscular dystrophies, immune deficiencies, blood, ocular and liver diseases. These products are developed either with Genethon as sponsor, or in partnership with private companies and academic institutions.

www.genethon.fr/en

Lysogene is a global biotechnology company, a leader in the basic research and clinical development of gene therapy for neurodegenerative disorders. Its mission is to radically improve the health of patients suffering from incurable life-threatening conditions by developing AAV vectors that have demonstrated their effectiveness in safely delivering genetic material to the central nervous system. Come and visit us at booth 16.

www.lysogene.com

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. Come and visit us at booth 9.

www.liebertpub.com/hum

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Oncorus, Inc. is an early-stage biotechnology company developing a next-generation immunotherapy platform to treat cancer. Oncorus's technology platform, based on innovative advancements with oncolytic viruses, has the potential to treat many tumor types, including highly malignant and aggressive cancers. Oncorus was founded by leading academic scientists and biotechnology entrepreneurs, including Mitchell H. Finer, PhD, an industry veteran and Managing Director of MPM Capital. A leader in corporate philanthropy, Oncorus has taken a pledge to donate a portion of product sales to fund promising cancer research and to support cancer care in the developing world. Oncorus is headquartered in Kendall Square, Cambridge, Massachusetts.

www.oncorus.com

Sangamo BioSciences, Inc. is focused on the development of genetic therapies based on its zinc finger protein (ZFP) technology platform for genome editing and gene regulation, and its AAV-cDNA gene therapy platform. In 2016, the company expects to initiate a Phase 1/2 clinical trial for its zinc finger nuclease (ZFN)-based therapeutic for the treatment of haemophilia B, which represents the first *in vivo* genome editing application in man. Sangamo also plans to file an Investigational New Drug (IND) application to initiate a Phase 1/2 clinical trial for haemophilia A based on its AAV-cDNA gene therapy approach. In addition, the company is developing ZFN-based therapeutics for lysosomal storage disorders, including MPS I (Hurler syndrome) and MPS II (Hunter syndrome), and has strategic collaborations with Biogen Inc. to develop therapeutics for sickle cell disease and beta-thalassemia, and with Shire International GmbH for Huntington's disease.

www.sangamo.com

ADVANCING Gene Therapy

Through ground-breaking collaborations and cutting-edge science, Biogen is identifying and developing new technologies for gene transfer and genome engineering.

Founded in 1978, Biogen is one of the world's oldest independent biotechnology companies. We are committed to advancing gene therapy.

To learn more about our vision for gene therapy visit WWW.BIOGEN.COM/BIOGENSCIENCE

Biogen

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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

Aldevron is a recognised leader in contract manufacturing and development services for nucleic acids, proteins and antibodies. Founded in 1998, we provide companies with essential components for research, clinical and commercial applications. Our products have supported numerous programmes in gene therapy, cell therapy and regenerative medicine from the bench to the bedside. Aldevron's services include GMP-Source™ and GMP plasmid manufacturing, linear DNA and mRNA production, gene synthesis, RNA synthesis enzymes and fully human and recombinant antibody generation. Our collaborative approach and commitment to providing quality materials allow us to meet precise client requirements and provide innovative solutions to advance science. Aldevron's headquarters is in Fargo, North Dakota and has facilities in Madison, Wisconsin and Freiburg, Germany.

www.aldevron.com

Brammer Bio is a contract development and manufacturing organisation (CDMO) serving companies seeking to develop and commercialise cell and gene therapies. With an experienced management team and exceptional scientific expertise and proven manufacturing capabilities we offer the knowledge and resources necessary to help you deliver innovative cell and gene-based therapies.

www.brammerbio.com

Headquartered in Parma, Italy, Chiesi Farmaceutici is an international research-focused Healthcare group, with over 80 years of experience in the pharmaceutical industry. Chiesi researches, develops and markets innovative drugs in the respiratory therapeutics, specialist medicine and rare diseases areas. Its R&D centres in Parma (Italy), Paris (France), Cary (USA), Chippenham (UK) and the R&D team of the acquired Danish company Zymenex, integrate their efforts to advance Chiesi's preclinical, clinical and registration programmes. Chiesi employs over 4,500 people, 560 of whom are solely dedicated to Research and Development activities.

www.chiesigroup.com

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Cobra Biologics is a leading international clinical and commercial manufacturer of biologics and pharmaceuticals with three GMP approved facilities. We offer a broad range of integrated and stand-alone development services, stretching from cell line development through to the commercial supply of investigational medicinal products. We take pride in manufacturing excellence and being a trusted provider, delivering what we promise and helping our customers to develop drugs for the benefit of patients. Cobra Biologics provides manufacturing solutions to the biologics and pharmaceutical industry.

www.cobrabio.com

EUFETS (Germany) is a Contract Development and Manufacturing Organisation specialising in the industrialisation of cell and gene therapy products (viral vectors, cell products and *in vitro transcribed* mRNA). Based on extensive expertise in molecular biology, virology and cell biology as well as an understanding of the regulatory prerequisites, our GMP experts support you to develop and manufacture your products in a safe and cost-efficient way. We offer a complete service spectrum from process and assay development through clinical trial medication to in-market supply in our state-of-the-art GMP facility. Come and visit us at booth 14.

www.eufets.com

GenoSafe is a CSO specialising in the evaluation of the quality, efficacy and safety of gene and cell therapy products. We offer 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. Come and visit us at booth 3.

www.genosafe.org

The flagship journal of the American Society of Gene and Cell therapy, *Molecular Therapy* is dedicated to publishing important peer-reviewed research and cutting-edge reviews and promoting the sciences in genetics, medicine and biotechnology. It is the parent journal to the open access titles *Molecular Therapy – Methods & Clinical Development*, *Molecular Therapy – Nucleic Acids*, and *Molecular Therapy – Oncolytics*.

www.nature.com

SILVER PARTNERS

Orchard Therapeutics is a clinical-stage biotechnology company with operations in London and the United States. We are dedicated to bringing transformative gene therapies to patients with serious and life-threatening orphan diseases.

www.orchard-tx.com

Oxford Genetics is a specialist synthetic biology company focused on providing DNA, protein, virus and cell line solutions for mammalian expression and bio-production. Our team of DNA designers and genetic engineers have access to a wide range of bioinformatics tools, novel technologies and pre-validated DNA sequences to help design, engineer, and deliver your project.

www.oxfordgenetics.com

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STEMCELL Technologies Inc is a privately owned biotechnology company that develops specialty cell culture media, cell separation products and ancillary reagents for life science research. Driven by science and a passion for quality, STEMCELL delivers over 1,500 products to more than 70 countries worldwide.

www.stemcell.com

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Age-Related Macular Degeneration (AMD) is a rapidly progressing, blinding disease that appears to result from age-associated alterations that include cell degeneration and vessel growth through Bruch's membrane into the subretinal space. Today's treatment includes repeated, frequent injections of VEGF (Vascular Endothelial Growth Factor) antibodies. PEDF (Pigment Epithelium-Derived Factor) as a physiological antagonist of VEGF should also inhibit the pro-angiogenic acting VEGF. The overall objective of TargetAMD is to deliver PEDF by using the hyperactive *Sleeping Beauty* (*SB100X*) transposon system in a cell-based, non-viral gene therapy in a clinical phase Ib/IIa trial.

www.targetamd.eu

uniQure

uniQure is a leader in the field of gene therapy. uniQure's Glybera, a gene therapy for the treatment of lipoprotein lipase deficiency, was the first approved gene therapy in the Western world. Using an innovative, modular technology platform, including our proprietary manufacturing process, uniQure is now advancing a broad pipeline of innovative gene therapies for diseases in the liver/metabolism, central nervous system, and cardiovascular areas, with an initial focus on treatments for rare diseases. In addition, through our collaborations and strategic partnership, we are making the next step towards developing gene therapies targeting chronic and degenerative diseases that affect larger populations.

www.uniqure.com

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- Cancer, immunomodulation and tissue regeneration

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- Product and process development, GMP manufacturing
- GMP-certified since 2010

www.apceth.com

Genenta Science develops a gene transfer strategy into autologous hematopoietic stem cells (HSCs) to target interferon-α expression to tumor-infiltrating monocytes/macrophages. An HIV-derived and genetically disabled viral vector – Lentivirus – delivers the gene into the HSCs. Type I Interferons have been shown to promote tumor immunity, but systemic toxicity has limited their use. The innovative therapy of Genenta Science, by combining transcriptional and microRNA-mediated control, enables tumor-infiltrating monocytes/macrophages to selectively express interferon-α limited to the tumor area, thus reducing its toxicity.

www.genenta.com

Sanofi Genzyme, the specialty care global business unit of Sanofi, focuses on rare diseases, multiple sclerosis, oncology, and immunology. We help people with debilitating and complex conditions that are often difficult to diagnose and treat. Our approach is shaped by our experience developing highly specialised treatments and forging close relationships with physician and patient communities. We are dedicated to discovering and advancing new therapies, providing hope to patients and their families around the world.

www.sanofigenzyme.com

BRONZE PARTNERS

TiGenix – an advanced biopharmaceutical company focused on developing and commercialising novel therapeutics from its proprietary platforms of allogeneic, or donor-derived, expanded stem cells. Two products from the adipose-derived stem cell technology platform are currently in clinical development. Cx601 is in Phase III for the treatment of complex perianal fistulas in Crohn's disease patients. In July 2016, TiGenix entered into a licensing agreement with Takeda, for the rights to Cx601 outside the United States. Cx611 has completed a Phase I sepsis challenge trial and a Phase I/II trial in rheumatoid arthritis. In 2015, TiGenix acquired Coretherapix, whose lead product, AlloCSC-01, is currently in a Phase II clinical trial in acute myocardial infarction (AMI). The second product candidate from the cardiac stem cell-based platform is AlloCSC-02, is being developed in a chronic indication. TiGenix is based in Leuven, Belgium, and has operations in Madrid, Spain.

www.tigenix.com

POSTERS BY CATEGORY

Poster session 1 (odd numbers) presented on Wednesday 19 october 2016 18.30-20.30

Leonardo, Fra' Angelico, Paolo Uccello, Giotto and Ballatoi rooms

Poster session 2 (even numbers) presented on Thursday 20 october 2016 18.30-20.00

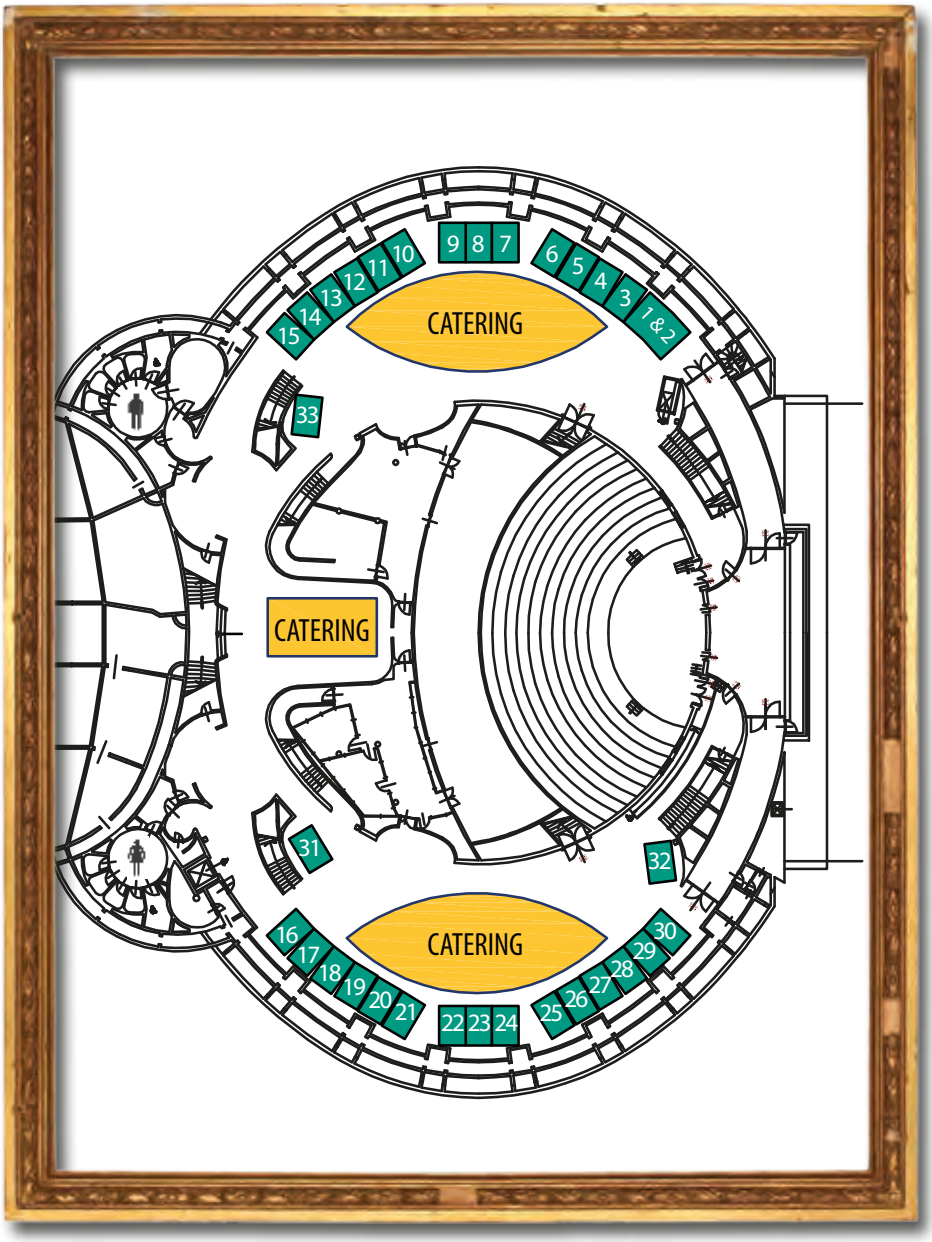
Leonardo, Fra' Angelico, Paolo Uccello, Giotto and Ballatoi rooms

PALAZZO CONGRESSI: BALLATOI (MEZZANINE LEVEL, ABOVE THE PASSI PERDUTI)	
P001-P021	Central nervous system gene therapy
P022-P037	Haematopoietic stem cells and homeostasis
P038-P040	Imaging stem cells dynamics
P041-P050	Immunology / cancer immuno-gene therapy
P051-P093	MSC gene and cell therapy
P094-P095	Organoids
P096-P114	Stem cell-based neural disease modelling
P115-P165	Cancer gene therapy

PALAZZO AFARI: 4TH FLOOR (PAOLO UCELLO AND GIOTTO ROOMS)	
P166-P173	Cancer stem cells
P174-P194	Cardiovascular gene and cell therapy
P195-P212	DNA-based gene transfer and integration studies
P213-P239	*Ex vivo* HSC-based gene and cell therapy

PALAZZO AFARI: 2ND FLOOR (FRA' ANGELICO AND LEONARDO ROOMS)	
P240-P263	Eye stem cell and gene therapy
P264-P276	Gene silencing
P277-P325	Genome editing and gene correction
P326-P329	Immunology and allergy
P330-P363	*In vivo* gene therapy
P364-P404	Manufacturing of cell and gene therapy products
P405-P430	Other developments
P431-P450	Other diseases
P451-P460	RNA-based gene transfer and integration studies

EXHIBITION HALL



1-2 gsk

3 GenoSafe

4 OXFORD GENETICS

5 LYSOGENE

6 cobra:bio
Expertise
Experience
Excellence

7 STEMCELL
VODEN medical instruments spa

8 EuroClone

9 FinVector Vision Therapies

10 Mary Ann Liebert, Inc. publishers

11 EBiSC

12 novasep

13 uniQure

14 EUFETS

15 PEPROTECH

16 COOK REGENTEC

17

18 ASEPTIC TECHNOLOGIES

19 MACS Miltenyi Biotec

20 MERCK

21 Lonza

22 SONY

23 CellGenix

24 cevec

25 BIO-RAD

26 DASIT GROUP CARLO ERBA

27 PALL Life Sciences

28 PlasmidFactory

29 ATCC LGC

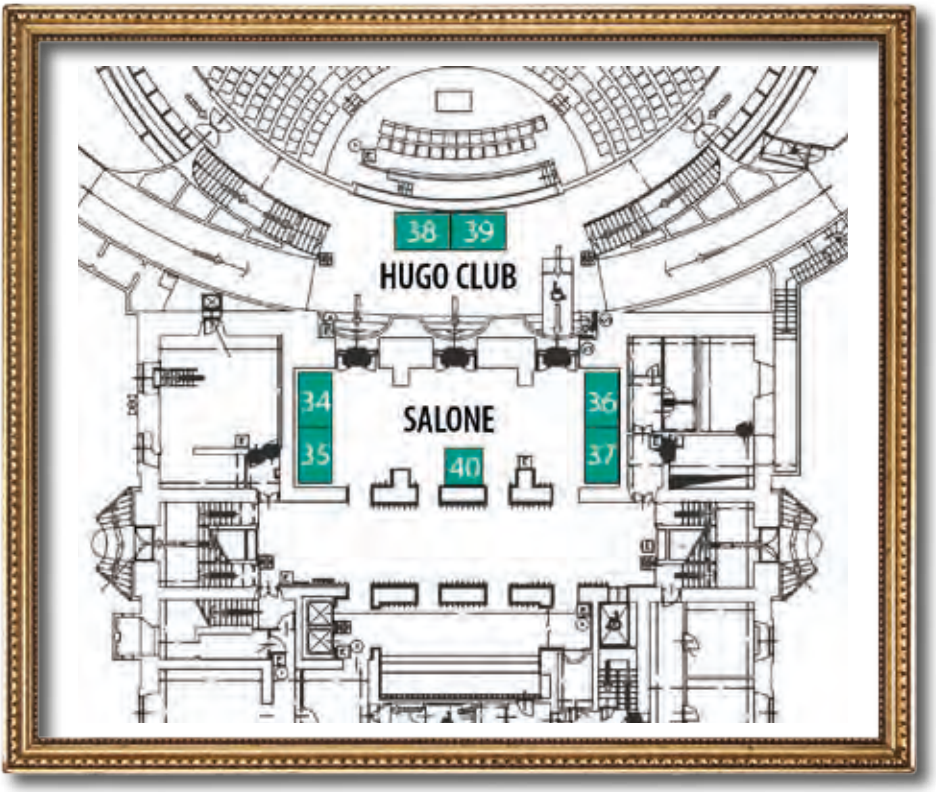
30 BioReliance by SAFC

31 chemometec

32 VIVEbioTECH

33 Cellular Dynamics International

EXHIBITION HALL



34 ALS Automated • Lab • Solutions

35 aldevron

36 geneWERK

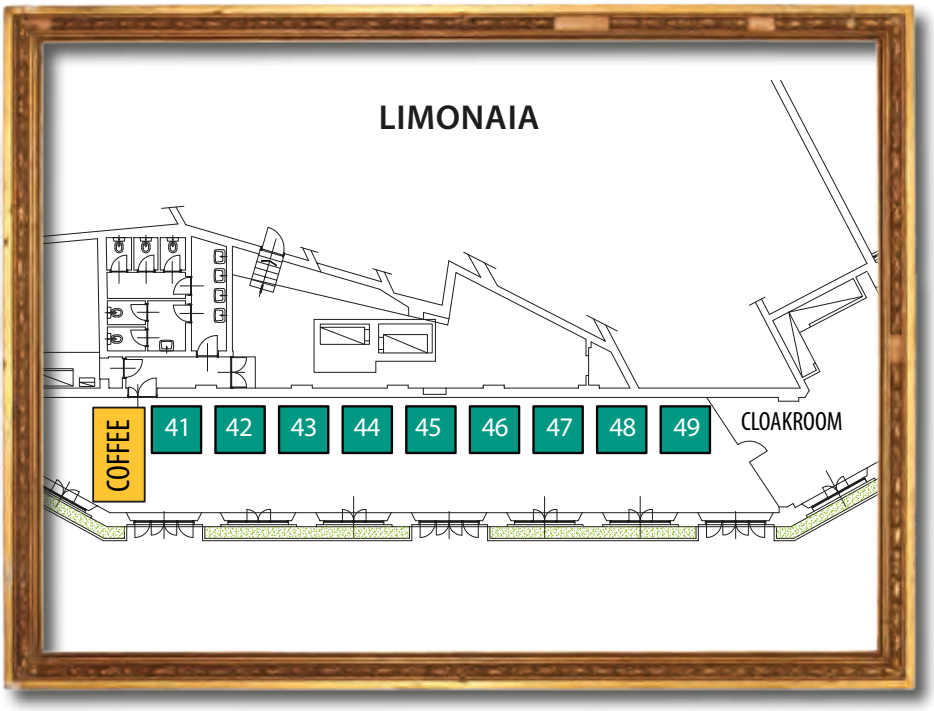
37 NHS Blood and Transplant

39 ALFA WASSERMANN

40 RuRo

38 PSN RESEARCH

EXHIBITION HALL



41 uSTEM

42 COVANCE SOLUTIONS MADE REAL

43 intellicyt A SARTORIUS COMPANY

44 brammer

45 CELL for CURE

46 BIOINVISION

47 DIM BIOTHERAPIES ilede France

48 EUROPEAN SOCIETY OF GENE & CELL THERAPY

49 ISSCR INTERNATIONAL SOCIETY FOR STEM CELL RESEARCH

EXHIBITORS

Booth 1–2: GlaxoSmithKline: see page 26
www.gsk.com

Booth 3: Genosafe: see page 37
www.genosafe.org

Booth 4: Oxford Genetics: see page 38
www.oxfordgenetics.com

Booth 5: Lysogene: see page 33
www.lysogene.com

Booth 6: Cobra Biologics: see page 37
www.cobrabio.com

Booth 7: STEMCELL Technologies: see page 38
www.stemcell.com

EXHIBITORS

Booth 8: EuroClone® is located in Italy. The Corporate Headquarters coordinate the activities of 2 satellite sites as well as the sales efforts of more than 70 Distributors worldwide, covering the most significant countries throughout 5 continents. EuroClone® is virtually able to meet all needs, in terms of reagents, equipment and know-how, which may arise in any of the following markets: Biotechnology and Diagnostics Medical Devices. The laboratory for Regenerative Medicine is the core of Euroclone group's R&D and includes scientists with expertise in cell biology, stem cells manipulation and development of protocols in compliance to GMP regulation. At the top of the range, particularly noteworthy, is the ISOCell PRO Cell Therapy Isolator. EuroClone®, with ISOCell, can be the answer to your needs by providing a streamlined workflow environment reducing the set up and running costs of cell therapy products preparation: a clean room in 1 m^2 leading regenerative medicine for everyone

www.euroclonegroup.it

Booth 9: FinVector: see page 32

www.finvector.com

Booth 10: *Human Gene Therapy*: see page 33

www.liebertpub.com/hum

Booth 11: Meet with representatives of the European Bank for induced pluripotent Stem Cells (EBiSC)! Discover the EBiSC iPSC Catalogue (https://cells.ebisc.org) and discuss how to best engage with EBiSC if you are interested in:

- ordering lines from the EBiSC catalogue;
- bio-sample procurement or depositing cell lines; and
- collaborating with EBiSC on future iPSC research projects.

At the EBiSC booth, we share our experience with you on:

- the establishment of an iPS cell bank with core and mirror facilities;
- the set-up of a robust and reliable supply chain for iPS lines including the generation of disease specific, control, gene edited and isogenic cell lines;
- standardised work flows from tissue procurement to generation, characterisation, preservation and supply;
- standardised quality control expansion; and
- the set-up of the ethical and legal governance structure for stem cell banking and distribution (informed consents, patient engagement, EBiSC MDA/AUA).

Find out more about this large European public-private partnership at:

www.ebisc.eu

EXHIBITORS

Booth 12: Novasep is a contract manufacturing organisation specialised in the production of viruses and viral vectors for gene therapy. Novasep offers specific know-how for culture and purification using the latest single-use and reusable technologies. From process development to the fill and finish step, Novasep is committed to the success of its customers.

www.novasep.com

Booth 13: uniQure: see page 39

www.uniqure.com

Booth 14: EUFETS: see page 37

www.eufets.com

Booth 15: 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, ELISA development kits, and other cytokine-related reagents.

www.peprotechec.com

Booth 16: Cook Regentec is focused on developing research and clinical tools to advance regenerative medicine therapies from the lab to the patient. Our team originated at Cook Medical, a medical device company that has worked with researchers and physicians for more than 50 years to develop more effective therapeutic tools. Cook Regentec's starting range of products includes cellular growth media, solutions for cryopreservation, and medical devices for the delivery of therapeutic agents.

www.cookregentec.com

Booth 17: TargetAMD: see page 39

www.targetamd.eu

EXHIBITORS

Booth 18: ASEPTIC TECHNOLOGIES provides a technology for cGMP aseptic fill and finish of ATMP. AT Closed Vial® technology consists in combination of:

- Ready-to-use closed vial (AT-Closed Vial®) ensuring container closure integrity during cryogenic storage;
- Filling equipment for small and extra small (less than 10 vials) batches easily installable in BioSafetyCabinet or Isolator.

Application of this technology in Regenerative Medicine enables better cell viability and recovery, reduction of residual volume, and quick operation with minimisation of contamination risks. The automated filling solutions are available for scaling-up. These are the main reasons why such companies as Celgene, Novartis, PCT, TissueGene, Celyad, TiGenix, CellforCure, Athersys, Stemedica, and UCL use it for their cell and gene therapy products.

www.aseptictech.com

Booth 19: Miltenyi Biotec 'From bench to bedside'

Miltenyi Biotec is Germany's largest independent, privately owned biotech company. Since pioneering MACS magnetic cell separation technology in 1990, we have grown into a vibrant, multinational team of more than 1,200 biomedical scientists, physicians, engineers and support groups. We develop and manufacture a portfolio of outstanding products ranging from unique cell labeling reagents, through sophisticated cell separation and analysis devices, to innovative systems for clinical applications. From research tools to GMP reagents for sophisticated applications, such as cellular therapy, the creativity of our interdisciplinary teams is reflected in the excellence of our products.

www.miltenyibiotec.com

Booth 20: Merck Millipore and Sigma-Aldrich come together, as Merck, to solve the toughest problems in life science by collaborating with the global scientific community. The life science business of Merck has a global network spanning more than 60 countries, approximately 70 manufacturing sites, 19,000 employees and over 1 million customers. The Company's portfolio of over 300,000 products can be viewed online - for more information, visit:

www.merckmillipore.com • www.sigma-aldrich.com

EXHIBITORS

Booth 21: 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 22: Sony Biotechnology Inc., part of the Sony Corporation, is a leading innovator of cell-based research systems. Sony Biotechnology is a total flow cytometry solutions provider supplying advanced and easy-to-use flow cytometry analysis and sorting technology for use in life science research. Sony Biotechnology's main goal is to develop and produce innovative products and techniques that dramatically improve the way researchers and scientists work. Recent products are the award-winning Sony SH800 Cell Sorter and Sony SP6800 Spectral Analyzer, as well as a complete line-up of over 8,000 reagents. Behind every Sony Biotechnology product is a team of experienced professionals who are dedicated to designing, manufacturing and supporting the highest quality products and most productive solutions for our customers.

www.sonybiotechnology.com

Booth 23: CellGenix is an international leading manufacturer and supplier of high-quality cytokines and serum-free medium for the *ex vivo* cell culture of DC, T-cells, NK-cells, hematopoietic stem cells, MSC, chondrocytes, ESC and iPS. CellGenix products are used worldwide in clinical trials in academia, commercial trials, production of vaccine and in translation, validation and testing or assay development by biotechnology partners. The manufacturing is in accordance with GMP guidelines and USP.

www.cellgenix.com

EXHIBITORS

Booth 24: CAP®GT is a regulatory endorsed expression platform for scalable viral vector production. CAP®GT suspension cells grow to high cell densities and show a broad viral propagation spectrum. Gene therapy vectors such as lentivirus (LV), adenovirus (AV) and adeno-associated virus (AAV) can be produced at industrial scale. CAP®GT enables better scale-up and competitive production costs when compared to adherent cell culture systems.

www.cevec.com

Booth 25: Bio-Rad Laboratories is a world leader in providing a broad range of products for the life science research and diagnostic markets. In our Life Science Group, we build the industry leading instruments, apparatus and consumables that enable advances in all key research areas from Cell Biology and Genomics through to Proteomics and Food Safety. Our innovative solutions include the pioneering Droplet Digital™ PCR alongside an extensive range of systems and reagents for qPCR, chromatography, cell analysis, immunoassay, electrophoresis, western blotting, imaging and more. For complete details of our comprehensive range of products contact your local office or visit our website:

www.discover.bio-rad.com

Booth 26: Carlo Erba Reagents, a privately owned company, was born in 2013 from the merger between two leading and complementary companies, Carlo Erba Reagenti S.p.A. and Dasit Sciences S.r.l. As a result, Carlo Erba, 160 years after its foundation, continues to support advancements and achievements in the fields of medicine and chemistry, now enriched by the Sciences Laboratory division, which serves as a 'partner of choice' of scientists. CARLO ERBA Reagents S.r.l. therefore follows a quasi-bicentennial tradition, with the ambition to offer to its customers innovative, customised products and services. Our experience ranges from Chemicals to Labware, from laboratory furniture and bio and chemical hoods to a Cell and Molecular Biology portfolio for high-tech applications in the Life Science field. CARLO ERBA Reagents is part of Dasit Group S.p.A., an Italian holding company founded in 1982 and owners of several well-known companies in the fields of In-Vitro Diagnostics, laboratory and industrial apparatuses for environmental protection (LAF) and laboratory ultrafreezers.

www.carloerbareagents.com

EXHIBITORS

PALL Life Sciences

Booth 27: Pall Corporation provides critical fluid management solutions to global life sciences and industrial manufacturing customers. The biopharm division of Pall Life Sciences features an unmatched portfolio of traditional and single-use products with custom service support from R&D to clinical phases to production. Pall is committed to continuously improving bioprocesses to enable users to advance global health with safe, environmentally responsible technologies. Stay up to date with our latest progress at: www.pall.com: LinkedIn; Twitter; and YouTube.

www.pall.com

PlasmidFactory

Booth 28: PlasmidFactory is Europe's leading contract manufacturer for plasmid DNA. Additionally, PlasmidFactory owns the essential rights to *minicircle* technologies worldwide. Production of plasmid and *minicircle* DNA ranges from research to industrial scale and is done in modern laboratories to the highest quality of standards and according to your individual wishes. Besides, PlasmidFactory holds an exclusive global licence for the manufacture and application for the *Helper & Packaging* plasmids of the pDG/pDP family by DKFZ Heidelberg, which are used in the production of AAV vectors. These plasmids enable simple and safe production of AAV vectors of different serotypes at high titres with only two plasmids co-transfected.

www.plasmidfactory.com

ATCC

LGC

Booth 29: ATCC provides reagents and services for cell therapy such as Stem Cell qualified serum for the culture of Mesenchymal Stem Cells, as well as a STR authentication service for the traceability and drift quality control of cultured MSCs before injection into patients. The ATCC collection also provides cellular models for your R&D projects such as human iPS and MSCs, human primary cells, hTERT-immortalised cell lines, and tumor cell lines. Coming soon: CRISPR-Cas9 engineered isogenic cell lines, iPSC-derived Neural Progenitor Cells and rodent primary neurons. LGC is the exclusive European distributor for ATCC's unique collections.

www.lgcstandards.com

BioReliance by SAFC

Booth 30: BioReliance / SAFC: see page 30

www.bioreliance.com

EXHIBITORS

Booth 31: ChemoMetec develops, manufactures and sells high quality automated Image Cytometer's within cell counters, which as the only ones on the market can count and analyse aggregated cells, adipose derived stem cells, cells growing on microcarriers with the highest precision. We also offer advanced cell analysers to help streamline processes for maximum efficiency. Our instruments are widely used in fields such as cancer research, stem cell research, production and quality control of a number of products such as pharmaceuticals, beer, animal semen and milk. We have specialised assays for aggregated cells, cells growing on microcarriers and adipose derived stem cells. 21 CFR Part 11 is also valued highly to have the highest standards. Our products are held in high regard because of their high quality and precision as well as the 'ease of use' advanced cell analysis. We value our customers; Therefore our policy is "no hidden costs" - no service agreements, high level of support and free software updates.

www.chemometec.com

Booth 32: VIVEbioTECH is a company specialising in gene transfer technologies and focused exclusively in the design and manufacture of lentiviral vectors for preclinical and clinical studies. The team has long experience in the development and manufacturing of lentiviruses for *in vitro* and *in vivo* research studies. The manufacturing process of lentiviral vectors has been optimised and the final yield increased significantly. Facilities, equipment and manufacturing protocols comply with the current GMP standards. VIVEbioTECH is client-oriented and highly flexible to their needs, being a one-stop company from lentiviral design to manufacturing and aseptic filling and finishing, highly competitive in prices and delivery times.

www.vivebiotech.com

Booth 33: CDI's mission is to advance the development of therapeutics for the most devastating human diseases by providing scientists with unparalleled access to biologically relevant human cells for use in drug discovery and cell therapy research. CDI employs more than 80 scientists with unparalleled experience in human stem cell culture and differentiation, genetic engineering, and process science. Using cutting-edge technologies, we have pioneered techniques for developing and manufacturing induced pluripotent stem (iPS) cells and differentiating them into functional human cells. CDI possesses the necessary intellectual property rights to produce and sell iPS cells and iPS cell-derived products and conveys a limited use license to its customers.

www.cellulardynamics.com

EXHIBITORS

Booth 34: ALS CellCelector™ is the only system which enables automated isolation of single cells, clusters, adherent cell colonies or colonies grown in 3D semi-solid media. It's an ideal system for (i) automated clonal picking of newly derived iPSC colonies, (ii) single cell or colony isolation for genome editing, and (iii) automated picking of hematopoietic stem cell colonies. Isolated colonies or single cells can be deposited into a variety of destination plates for downstream culturing or molecular characterisation (qPCR, sequencing…). CellCelector combines bright field, phase contract or fluorescence imaging, sensitive cell/colony detection technology and patented robotics picking tools. The system can be also used for stem cell culture monitoring and be integrated into a fully automated stem cell production facility.

www.als-jena.com

Booth 35: Aldevron. See page 36

www.aldevron.com

Booth 36: GeneWerk GmbH is a German startup company. The team has long-lasting experience in the area of hematology, oncology and virology with focus on integration site analysis, sequencing and bioinformatics. GeneWerk provides custom-tailored service based on 20 years of experience in the field of gene therapy, gene editing, immunotherapy and related areas.

www.genewerk.de

Booth 37: NHS Blood and Transplant (NHSBT) is a national organisation within the NHS dedicated to saving and improving lives through the wide range of services we provide to the healthcare community. The Cellular and Molecular Therapy (CMT) function of NHSBT offers broad experience and expertise in novel stem cell therapies, processing technologies and gene therapy-based treatments and research. We have three MHRA licensed Advanced Therapy Units providing GMP cell therapy manufacture and a further four laboratory sites with HTA licences. Our Clinical Biotechnology Centre specialises in the manufacture of plasmid DNA and novel recombinant proteins. MHRA licensed and fully GMP compliant, CBC operates from a production suite comprising multiple segregated rooms for processing and final fill. NHSBT offers strength in specialist manufacturing, scientific skills, translational experience, regulatory expertise and distribution in support of cellular and molecular therapies. We welcome partnerships with clinical, academic and commercial organisations within this developing field. cmt@nhsbt.nhs.uk

www.nhsbt.nhs.uk

EXHIBITORS

Booth 38: PSNResearch is a full service CRO focusing on small and medium sized biotech/pharma and providing clinical research services for all types of clinical studies. With over 230 highly experienced research professionals in 7 countries across the USA and the EU, PSNResearch is large enough to accommodate all clinical development programmes and specific projects, but small enough to provide personalised project specific solutions. PSNResearch is committed to making multinational studies more cost effective and successful. Clinical development of Advanced Therapy Medicinal Products is a rapidly expanding area and calls for experienced CROs with good knowledge of relevant regulations. PSNResearch has participated in 11 cell and gene therapy programmes in various disorders, and is aware that these studies require robust but flexible management; from initial toxicology studies through to human trials. PSNResearch is experienced in running these studies from initial regulatory scientific advice to final reports, in full compliance with regulatory requirements

www.psnresearch.com

Booth 39: Alfa Wassermann Separation Technologies is the leader in continuous flow ultracentrifugation solutions for process development and industrial scale manufacturing. AW products are used globally for viral vaccine and Gene Therapy Products in cGMP manufacturing facilities. AW provides full scale cGMP ultracentrifuges, KII, for production, PKII for pilot scale and also provides a laboratory scale ultracentrifuge. The AW Promatix 1000™ is the first fully automated laboratory Ultracentrifuge capable of continuous flow operations. The Ultracentrifuge is fully programmable to run user defined applications and is fully automated for gradient and product loading, continuous flow or batch separation and fraction collection and cleaning.

www.awst.com

Booth 40: Headquartered in the heart of Maryland's biotechnology corridor and with subsidiaries in China and Europe, RURO specialises in Laboratory Information Management Solutions for research, biotechnological, pharmaceutical, healthcare and government (homeland security) laboratories. RURO's Limfinity is the central data management solution in many of the world's leading Clinical Trials, Translation Science programmes and Biobanks. RURO's Radio Frequency Identification (RFID) Solutions meet critical inventory management, tracking and security needs for industrial and laboratories utilising select agents. Our recent line of biological applications for Rare Diseases is designed to increase the productivity of scientific, biotech and pharmaceutical laboratories while maintaining the highest level of security, versatility and knowledge. RURO is Laboratory Information Bliss!

www.ruro.com

EXHIBITORS

Booth 41: uSTEM provides an effective and transgene-free reprogramming service based on a proprietary microtechnology.

www.ustemcells.info

Booth 42: Covance Inc., the drug development business of Laboratory Corporation of America Holdings (LabCorp) headquartered in Princeton, New Jersey, USA, is the world's most comprehensive drug development company, dedicated to advancing healthcare and delivering Solutions Made Real®. Our unique perspectives, built from decades of scientific expertise and precision delivery of the largest volume of drug development data in the world, helps our clients identify new approaches and anticipate tomorrow's challenges as they evolve. Together with our clients, Covance transforms today's health care challenges into tomorrow's solutions. Information on Covance's solutions can be obtained through its website at:

www.covance.com

Booth 43: IntelliCyt Corporation develops integrated solutions for cellular analysis and virus quantitation that expand scientific discovery beyond current capabilities, enable physiologically-relevant experimental models, and enhance productivity to provide insight into complex disease states. The iQue Screener platform enables rapid, high content, multiplexed analysis of suspension cells and secreted proteins for immunology and immuno-oncology profiling, antibody discovery, and immune targets screening in drug discovery and translational research. The ViroCyt platform provides rapid virus quantification, delivering significant improvements to mission critical processes, such as vaccine manufacturing, protein expression, antiviral development and other settings where viruses play a significant role.

www.intellicyt.com

Booth 44: Brammer Bio. See page 36.

www.brammerbio.com

Booth 45: CELLforCURE. See page 32.

www.cellforcure.com

EXHIBITORS

Booth 46: BioInVision, based in the USA, offers imaging instrumentation and methodologies critical to preclinical studies. The unique CryoViz instrument, utilising the patented cryo-imaging technology, allows microscopical anatomical and molecular fluorescence imaging of laboratory small animals such as a mouse or organs excised from them with single-cell sensitivity. With its sub-10-micron-scale imaging, cryo-imaging allows one to detect even single stem or cancer cells anywhere in a mouse. The technology is also offered as a service and is targeted to a variety of biomedical applications including stem cell homing and biodistribution, cancer metastatis, imaging agents, drug discovery, tissue engineering, mouse phenotyping etc.

www.bioinvision.com

Booth 47: The DIM* Biotherapie is a scientific network sponsored by the Paris Ile de France Region to support, develop and structure research in the field of Regenerative Medicine: Gene Therapy, Cell therapy, Stem Cell Research, Developmental Biology and Transplantation. Through annual call-for-proposals, the Dim Biotherapy finances doctoral contract salaries (opento European students), laboratory small and large equipment and scientific workshops in The Paris Ile de France region.

**DIM are 'Domaines d'Intérêt Majeur,' (Major Interest Domains) that were identified (2012–2017) by the French Region 'Ile de France' including Paris and its surroundings, to foster and to develop scientific research programmes in strategic fields.*

www.dim-biotherapies.com/en/

Booth 48: Drop into the ESGCT booth for all you need to know about ESGCT. This is the place to come if you have any questions about this year's congress, future congresses and ESGCT, including:

- buy tickets for this year's Molecular Mingle (cash only)
- information about the 2017 congress in Berlin
- social media hub – play games and win prizes including a limited number of free Berlin Molecular Mingle tickets
- collect your Molecular Mingle drinks vouchers
- information about the ESGCT Spring School in Granada
- ESGCT membership information.

www.esgct.eu

Booth 49: Visit the International Society for Stem Cell Research (ISSCR) stand. Learn more about upcoming ISSCR meetings, membership benefits and our online webinars. All attendees to this meeting will be invited to join the ISSCR. Be a part of the leading association for the global stem cell research community.

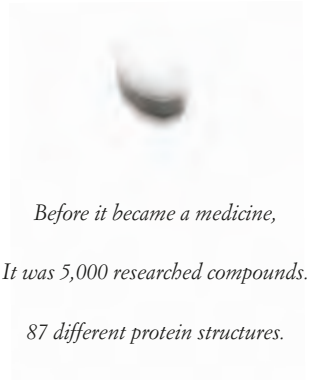
www.isscr.org

FULLY COMMITTED
to the fight against SMA

At AveXis, we are working relentlessly to bring gene therapy to patients and families affected by rare genetic diseases. Our initial focus is on spinal muscular atrophy (SMA) Type 1— the leading genetic cause of infant mortality, which currently has no FDA-approved therapy. That's why we are pushing forward with the clinical development of AVXS-101 for the treatment of SMA.

For more information about AveXis,
please visit www.avexis.com.

avexis



Before it became a medicine,

It was 5,000 researched compounds.

87 different protein structures.

500,000 lab tests.

1,600 scientists.

80-hour workweeks.

14 years of breakthroughs and setbacks.

36 clinical trials.

8,500 patient volunteers.

And more problems to solve than we could count.

Before it became a medicine,

It was an idea in the mind of a Pfizer scientist.

Now it's a medicine

That saves lives every day.

Pfizer

Driven to discover the cure

THE MOLECULAR MINGLE

JOIN US FOR AN EVENING OF NETWORKING AND ARTISAN FOOD AT THE MERCATO CENTRALE

Mercato Centrale Firenze is a large covered marketplace where authenticity, spontaneity and tradition play a leading role.

Opened in 2014 on the first floor of the historic iron and glass building designed in 1874 by Giuseppe Mengoni, the market has revived the San Lorenzo neighbourhood, providing traditional shops that give food artisans centre stage. Bread and confectionary, fresh fish, fried food and rissoles, fruit and vegetables, meats and salamis, buffalo mozzarella, cheese, chocolate and ice cream, fresh pasta, wines, lampredotto and sandwiches: all shops are run by traders who share a passion for their craft.

For one evening, on 20 October 2016, the market will be open for exclusive use by Congress delegates.

www.mercatocentrale.it

Thursday 20 October, 8.30pm–1am
Live band 8.30–11pm, DJ set on the terrace 11pm–1am

The Mercato Centrale is a five minute walk from the Palazzo Congressi

Tickets still available to purchase at the ESGCT booth
€50 per person
2 drinks vouchers per person to be collected from the ESGCT booth, see page 59


I FORMAGGI
IL VINO
IL PANE
E LA PASTICCERIA
LE BOTTEGHE
GLI ARTIGIAN

PROGRAMME

TUESDAY 18 OCTOBER 2016

CLINICAL TRIAL AND COMMERCIALISATION WORKSHOP *First floor: Piero della Francesca room*	
08.00-09.00	Registration
09.00-09.20	**Planning a clinical trial**
09.00-09.20	INV001 Considerations for clinical trials with cellular therapies *Kim Champion, University College London*
09.20-10.00	**Manufacturing of gene and cell products**
09.20-09.40	INV002 What should be anticipated and implemented to ensure a successful process transfer. A CMO perspective with risk analysis from analytical to process transfer *Francis Dupont, Novasep, Gosselies*
09.40-10.00	INV003 Development and manufacture of iPSC-derived cells to a specification: the CDI experience as a contractor *Derek Hei, Cellular Dynamics International, Madison, WI*
	Coffee available in the room
10.00-10.20	**Gene and cell therapy technologies**
10.10-10.20	INV004 Gene and cell therapy technologies *Bobby Gaspar, University College London*
10.20-11.20	**Pricing and reimbursement**
10.20-10.40	INV005 Early insights from NICE: ATMPs – evidence generation, evaluation, managed access *Leeza Osipenko, NICE, London*
10.40-11.00	INV006 Hurdles in developing new Advanced Therapies: the experience with Holoclar *Andrea Chiesi, Chiesi, Parma*
11.00-11.20	INV007 The Strimvelis experience *Claude Schmitt, GSK, Brentford*
11.20-11.40	**Academic vs commercial clinical development strategy**
11.20-11.40	INV008 Running clinical trials through a venture capital financed biotech company *Kerry Fisher, University of Oxford*

PROGRAMME

TUESDAY 18 OCTOBER 2016

CLINICAL TRIAL AND COMMERCIALISATION WORKSHOP *First floor: Piero della Francesca room*	
11.40-12.00	**Regulatory strategy in gene and cell therapy development** *Chair: Guido Pantè, AIFA*
11.40-12.00	INV009 Scientific and regulatory challenges for developing advanced therapy medicinal products in EU *Maria Cristina Galli, ISS, Rome*
12.00-12.40	**Finding the value**
12.00-12.20	INV010 Developing innovative treatments for rare diseases: the Telethon Foundation model *Lucia Faccio, Telethon Foundation, Milan*
12.20-12.40	INV011 Molecular therapies targeting Huntington: progress and challenges *Ignacio Munoz-Sanjuan, CHDI Foundation Inc, Los Angeles, CA*
12.40-13.00	**Elevator pitches**
12.40-12.45	EL001 Gene transfer technologies *Juan Carlos Ramírez, Vivebiotech, San Sebastian*
12.45-12.50	EL002 Development of advanced therapy medicinal products: need of specialised regulatory services support *Ana Belen del Campo, PSNResearch, Madrid*
12.50-12.55	EL003 Orchard Therapeutics *Andrea Spezzi, Orchard Therapeutics, London*
12.55-13.00	EL004 A leap forward in AAV research and development *Sven Kuhlendahl, Progen, Heidelberg*

PROGRAMME

TUESDAY 18 OCTOBER 2016

EDUCATION DAY *-1 floor Michelangelo*	
08.00-09.00	Registration
09.00-09.30	**E1: Opening words** *Chairs: Sam Wadsworth, Hildegard Büning*
09.00-09.05	Introduction *Sam Wadsworth, Dimension Therapeutics, Cambridge, MA*
09.05-09.30	INV012 Brief introduction and update on recent developments in cell and gene therapy *Hildegard Büning, University of Cologne, DZIF, University Hospital Cologne, Hannover Medical School*
09.30-10.30	**E2a: Tailoring gene transfer vectors**
09.30-10.00	INV013 Improving the efficacy of gene therapy vectors by *de novo* design of transcriptional cis-regulatory modules: implications for gene therapy and CRISPR/Cas9-mediated gene editing *Thierry VandenDriessche, Free University of Brussels; University of Leuven*
10.00-10.30	INV014 Receptor-targeted viral vectors *Christian Buccholz, Paul-Ehrlich-Institut Langen*
10.30-11.00	**E2b: Disease modelling**
10.30-11.00	INV015 p63 as a master regulator of epithelial stemness, identity, and integrity *Caterina Missero, University of Naples Federico II; Center for Genetic Engineering*
11.00-11.30	Coffee Break
11.30-12.30	**E3: Stem cells and iPS – current state**
11.30-12.00	INV016 Reprogramming of somatic cells for studies of liver diseases *Tobias Cantz, Hannover Medical School*
12.00-12.30	INV017 *In vitro* modelling of human neocortical development process using pluripotent stem cells: from neural lineage induction to neuronal subtype specification *Luciano Conti, Centre for integrative Biology, University of Trento*
12.30-13.30	Lunch – *Limonaia, Passi Perduti*

PROGRAMME

TUESDAY 18 OCTOBER 2016

EDUCATION DAY *Brunelleschi Auditorium*	
13.30-14.30	**E4a: Immunotherapy and transdifferentiation**
13.30-14.00	INV018 Clinical pharmacology of CAR-T cells *Attilio Bondanza, San Raffaele Scientific Institute, Milan*
14.00-14.30	INV019 Streamline cell reprogramming by direct conversion of fibroblasts into neurons and glia: hurdles and opportunities *Vania Broccoli, San Raffaele Scientific Institute, Milan*
14.30-15.30	**E4b: Gene editing**
14.30-15.00	INV020 Gene edited stem cells: from cloning to clinic *Jakub Tolar, University of Minnesota*
15.00-15.30	INV021 Genome editing using CRISPR-Cas nucleases *Keith Joung, Massachusetts General Hospital; Harvard Medical School*
15.30-16.00	Coffee break

PROGRAMME

TUESDAY 18 OCTOBER 2016

PUBLIC ENGAGEMENT DAY FOR PATIENT ASSOCIATIONS AND SCIENCE DIALOGUES WITH CITIZENS: **Leading edge therapies for rare diseases** *First floor, Masaccio room*	
09.00-09.45	Registration
09.45-10.15	**Gene therapy**
09.45-10.15	Opening lecture *Alberto Auricchio, Tigem, Naples*
10.15-10.45	**How to foster access to therapies**
10.15-10.45	Newborn screenings for metabolic diseases *Giancarlo La Marca, University of Florence*
10.45-11.15	**Safety studies**
10.45-11.15	Use of animal models in research: why it is still a need *Giuliano Grignaschi, Istituto Di Ricerche Farmacologiche Mario Negri, Milan*
11.15-11.45	**Science and bio-ethics**
11.15-11.45	Stem cells and new generation sequencing: new frontiers in medicine *Giuseppe Testa, European Institute of Oncology, University of Milan*
11.45-13.00	**Discussion** *Panelists:* *Nicola Spinelli Casacchia, President, Uniamo FIMR* *Andrea Buzzi, President, Fondazione Paracelso* *Michele Lipucci, Eurordis* *Chairs:* *Ilaria Bartoli Ciancaleoni, Osservatorio Malattie Rare* *Alessia Daturi, Telethon Foundation*
13.00-14.00	Lunch and networking (in the room)
14.00-14.30	**New frontiers in science**
14.00-14.30	Gene editing, a new era in molecular biology *Luigi Naldini, SR-Tiget, Milan*
14.30-16.30	**Role playing: science dialogues** *Chair: Anna Maria Zaccheddu, Telethon Foundation*
16.30-17.00	**Closing remarks**

PROGRAMME

TUESDAY 18 OCTOBER 2016

MOLMED SYMPOSIUM

An entrepreneurial approach to translate academic knowledge into therapeutic solutions for all patients

Ground floor, Botticelli room

13.00-14.00	Registration
14.00-14.15	When pioneers in cell and gene therapy come up with a 'business' idea *Claudio Bordignon, Molmed, Milan*
14.15-14.30	Academia ready to be a productive partner for biotech companies *Fabio Ciceri, San Raffaele Scientific Institute, Milan*
14.30-14.45	How the financial market operated and operates in sustaining the biotech sector development *Gil Bar-Nahum, Jefferies Global Healthcare Group, Bresso*
14.45-15.00	A picture of the European biotech sector: strengths and weaknesses *Carlo Incerti, EuropaBio, Bresso*
15.00-15.30	Round Table ***Moderator: Frediano Finucci (La7)*** *Martin Andrews, GSK, Brentford* *Gil Bar-Nahum, Global Healthcare Group at Jefferies International Limited* *Claudio Bordignon, MolMed, Milan* *Fabio Ciceri, San Raffaele Scientific Institute, Milan* *Carlo Incerti, EuropaBio, Bresso* *Francesca Pasinelli, Telethon Foundation*

PROGRAMME

TUESDAY 18 OCTOBER 2016

MAIN CONGRESS	
16.00-17.00 *Auditorium*	**ESGCT / ISSCR 2016 Opening: welcome and introduction and Opening Keynote** *Chairs: Nathalie Cartier-Lacave, Luigi Naldini* bluebirdbio
16.00-16.15	Welcome *Nathalie Cartier-Lacave, ESGCT; Luigi Naldini, Local Organising Committee; Nancy Witty, ISSCR*
16.15-17.00	INV023 Lgr5 stem cell-grown organoids and their applications *Hans Clevers, University Medical Centre Utrecht and Princess Maxima Center for pediatric oncology, Utrecht*
17.00-19.00 *Auditorium*	**1: Neural diseases: modelling, reprogramming and transplantation in brain and retina** *Chairs: Giuseppe Testa, Vania Broccoli* REGENXBIO
17.00-17.30	INV024 Modelling human psychiatric disease *Fred Gage, The Salk Institute, La Jolla, California*
17.30-18.00	INV025 Retinal cell using iPS cells *Masayo Takahashi, RIKEN, CDB, Kobe*
18.00-18.30	INV026 Towards a stem cell-based therapy for Parkinson's disease *Malin Parmar, Lund University*
18.30-19.00	INV027 Chemical approaches to oligodendrocyte remyelination *Paul Tesar, Case Western Reserve University, Cleveland, OH*
19.00-20.00 *Limonaia, Passi Perduti*	Welcome reception
19.00-21.00 *Ucello room*	Molecular therapy 'meet the editor' reception Molecular Therapy

PROGRAMME

WEDNESDAY 19 OCTOBER 2016

MAIN CONGRESS	
08.30-10.30 *Auditorium*	**2: Hematopoietic stem cells: from biology to clinical applications** *Chairs: George Q Daley, Alessandro Aiuti*
08.30-09.00	INV028 How is human blood made? *John Dick, Princess Margaret Cancer Centre, University Health Network, University of Toronto*
09.00-09.30	INV029 Using zebrafish to find new therapies for blood diseases *Leonard Zon, Boston Children's Hospital, Harvard Medical School*
09.30-10.00	INV030 Advanced genetic engineering of hematopoiesis to treat human diseases *Luigi Naldini, SR-Tiget, Milan*
10.00-10.30	INV031 Gene therapy of inherited disease: advances and challenges *Marina Cavazzana, Hôpital Universitaire Necker, Enfants Malades, Paris*
10.30-11.00 *Limonaia, Passi Perduti*	Coffee break
	Parallel sessions 2a, 2b, 2c
11.00-12.30 *Botticelli*	**2a: Imaging stem cells dynamics** *Chairs: Fred Gage, Dominique Bonnet*
11.00-11.30	INV032 Tissue-scale coordination of cellular homeostatic and repair behaviors in live mice *Valentina Greco, Yale University*
11.30-12.00	INV033 Long-term single cell quantification: new tools for old questions *Timm Schröder, ETH Zürich*
	Proffered papers
12.00-12.15	OR001 Altered functional activity in vmDA neurons derived from Parkinson's disease-induced pluripotent stem cells (iPSC) *Giulia Carola, Institute of Biomedicine of the University of Barcelona (IBUB)*
12.15-12.30	OR002 3D-imaging and tissue reconstruction of deep-brain gene silencing with nanoscale, non-viral siRNA complexes *Yein Nam, University of Manchester*
11.00-12.30 *Masaccio*	**2b: Eye stem cell and gene therapy** *Chairs: Robin Ali, Alberto Auricchio*
11.00-11.30	INV034 What does influence regeneration? *Graziella Pellegrini, Center for Regenerative Medicine, University of Modena*

PROGRAMME

WEDNESDAY 19 OCTOBER 2016

11.30-12.00	INV035 AAV mediated gene therapy and beyond - maintaining and restoring vision *Deniz Dalkara, Inserm, UPMC Paris 6*
	Proffered papers
12.00-12.15	OR003 Generation and transplantation of human pluripotent stem cell derived cone photoreceptors into models of retinal degeneration *Anai Gonzalez Cordero, University College London*
12.15-12.30	OR004 One-year follow-up study results after Intravitreal rAAV2/2-ND4 (GS010) injection in patients with vision loss due to G11778A ND4 Leber Hereditary Optic Neuropathy *Jean-Philippe Combal, Gensight, Paris*
11.00-12.30 *Auditorium*	**2c: Central nervous system gene therapy** *Chairs: Nathalie Cartier-Lacave, Jerry Mendell*
11.00-11.30	INV036 HSC-based cell and gene therapy approaches for treating LSDs *Alessandra Biffi, Gene Therapy Programme, Dana-Farber/Boston Children's Cancer and Blood Disorders Center*
11.30-12.00	INV037 Gene therapy for neurodegenerative diseases *Shin-ichi Muramatsu, Division of Neurology, Department of Medicine Jichi Medical University*
	Proffered papers
12.00-12.15	OR005 Survival of embryonic tissue grafts in Parkinson's disease: neuroimaging and clinical evidence at 17-18 years post-transplant *Claire Henchcliffe, Weill Cornell Medical College, NY*
12.15-12.30	OR006 PGC-1α overexpression by lentiviral vector attenuates amyloid-β load and neuronal loss in an Alzheimer's disease model *Nick Mazarakis, Imperial College London*
12.30-14.00 *Limonaia, Passi Perduti*	Lunch *Posters available for viewing in Leonardo, Fra' Angelico, Paolo Uccello, Giotto and Ballatoi rooms*
12.45-13.45 *Michelangelo*	**Lunch Symposium: Regulatory workshop for ATMPs** (Lunch is available in the room)
12.45-13.15	Regulatory guidance and advice on the quality control of Advanced Therapy Medicinal Products *Martin Wisher, BioReliance*
13:15 - 13:45	A cell and gene therapy registry: a proposal from EBMT *Chiara Bonini, Università Vita-Salute San Raffaele and Ospedale San Raffaele Scientific Institute, Milan*

PROGRAMME

WEDNESDAY 19 OCTOBER 2016

14.00-16.00 *Auditorium*	**3: Skeletal and cardiac muscle stem cells: from biology and reprogramming to clinical applications** *Chairs: Wim Fibbe, Fulvio Mavilio* *In memoriam Paolo Bianco*
14.00-14.30	INV038 Challenges of immaturity and proliferation in using hPSC-derived cardiomyocytes as disease models *Christine Mummery, Leiden University Medical Center, University of Twente*
14.30-15.00	INV039 Small RNA therapy for cardiac regeneration *Mauro Giacca, International Centre for Genetic Engineering and Biotechnology (ICGEB), Trieste*
15.00-15.30	INV040 Remuscularisation of injured hearts with human embryonic stem cell-derived cardiomyocytes *Michael Laflamme, University Health Network, Toronto*
15.30-16.00	INV041 Tricyclo-DNA: a new generation of antisense oligonucleotides for splice switching *Luis Garcia, Inserm UMR 1179, Paris*
16.00-16.30 *Limonaia, Passi Perduti*	Coffee break
	Parallel sessions 3a, 3b, 3c, 3d
16.30-18.30 *Botticelli*	**3a: Organoids and high throughput platforms** *Chairs: Valentina Greco, Melissa Little*
16.30-17.00	INV042 Generating 3D models of the human cerebral cortex to study development and disease *Sergiu Pasca, Stanford University, CA*
17.00-17.30	INV043 Detecting and killing pancreatic cancer *David Tuveson, Cold Spring Harbor Laboratory, NY*
	Proffered papers
17.30-17.45	OR007 Generation of implantable 3D skeletal muscle tissue from human embryonic stem cells and muscular dystrophy iPS cells *Francesco Saverio Tedesco, University College London*
17.45-18.00	OR008 The development and characterisation of rAAV vectors in patient-derived intestinal organoids and CF mice as a treatment for cystic fibrosis *Marianne Carlon, Laboratory for Molecular Virology and Drug Discovery, Division of Molecular Medicine, KU Leuven*
18.00-18.15	OR009 Isolating and characterising human cone photoreceptors for cell therapy *Emily Welby, University College London*

PROGRAMME

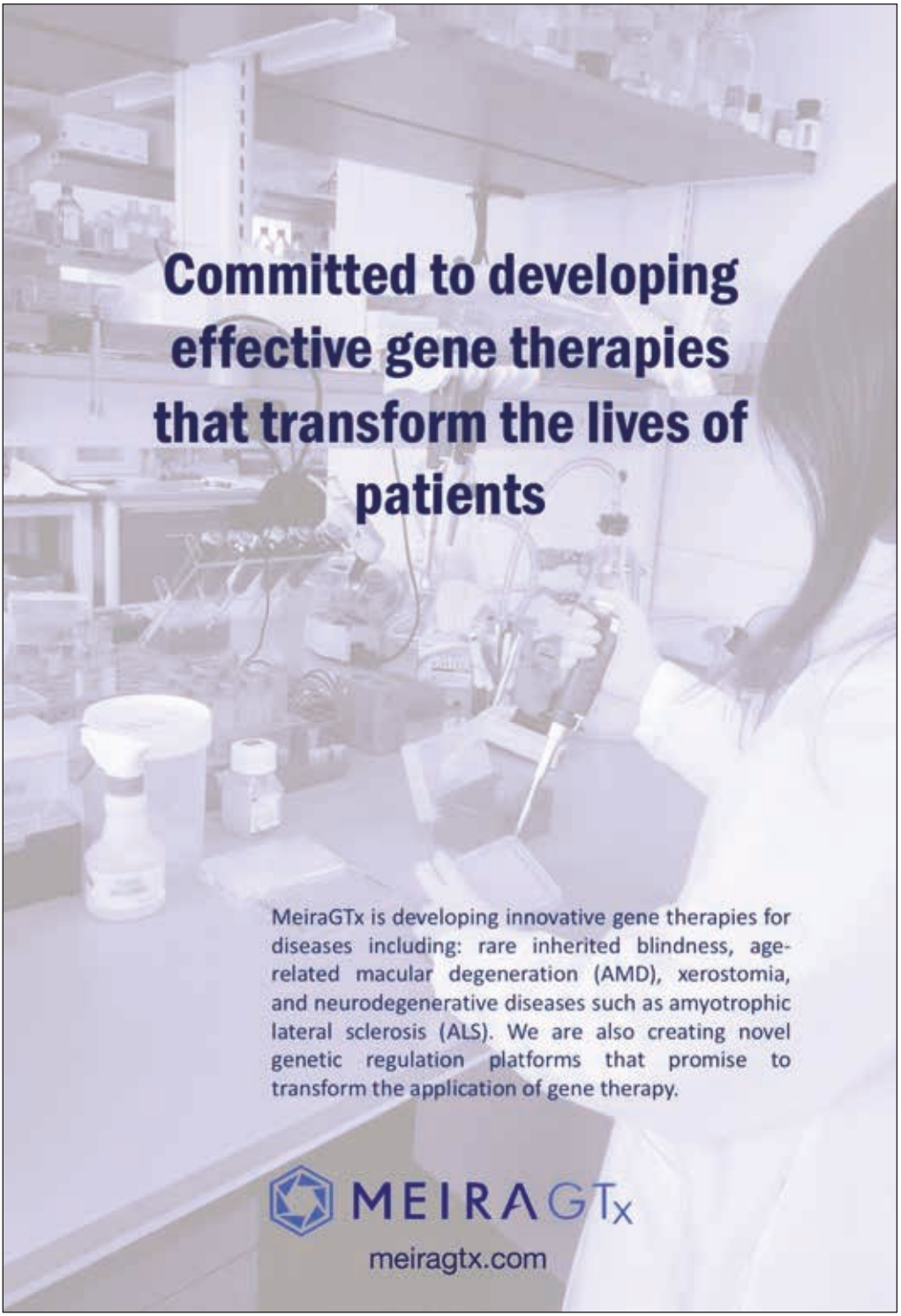
WEDNESDAY 19 OCTOBER 2016

18.15-18.30	OR010 Assay development and high throughput screening in iPSCs-derived cortical glutamatergic neurons from two neurodevelopmental disorders caused by symmetrical dosage imbalance *Francesca Cavallo, University of Milan*
16.30-18.30 *Masaccio*	**3b: Stem cell-based neural disease modelling** *Chairs: Angela Gritti, Paul Tesar*
16.30-17.00	INV044 Dissecting the genetic and environmental causes of chromatin dysfunction in autism and intellectual disability: an integrated platform of 2D and 3D stem cell-based models of neural development *Giuseppe Testa, European Institute of Oncology, University of Milan*
17.00-17.30	INV045 iPSC-based modelling of Parkinson's disease *Angel Raya, Center for Regenerative Medicine, Barcelona*
	Proffered papers
17.30-17.45	OR011 Two factor fibroblast reprogramming generates induced Schwann cells with myelinogenic and nerve regenerating potential *Pietro Mazzara, SR-Tiget, Milan*
17.45-18.00	OR012 Neural stem cell transplantation in Parkinsonian mice triggers an astrocyte-dependent dopaminergic neurorestoration *Bianca Marchetti, University of Catania*
18.00-18.15	OR013 Leveraging pluripotent stem cells as a scalable platform to discover chemical therapeutics for genetic disorders of myelin *Matthew Ellit, Case Western Reserve University, Cleveland, OH*
18.15-18.30	OR014 Mitochondrial disease phenotype in Friedreich's ataxia patient iPSC-derived sensory neurons *Roxana Natt, Medical University Innsbruck*
16.30-18.30 *Michelangelo*	**3c: Cardiovascular gene and cell therapy** *Chairs: Seppo Ylä-Herttuala, Mauro Giacca*
16.30-17.00	INV046 Therapeutic vascular growth for cardiovascular diseases *Seppo Ylä-Herttuala, AI Virtanen Institute, Kuopio*
17.00-17.30	INV047 Non-coding RNA in vascular repair and regeneration *Andrew Baker, The University of Edinburgh*
	Proffered papers
17.30-17.45	OR015 Fixing the so-called unfixable: regenerating untreatable fixed myocardial scar in heart failure patients *Ajan Reginald, Celixir Limited, Cardiff*
17.45-18.00	OR016 Using omics tools to improve differentiation and maturation of cardiomyocytes derived from human pluripotent stem cells *Paula Alves, iBET/ITQB, Oeiras*

PROGRAMME

WEDNESDAY 19 OCTOBER 2016

18.00-18.15	OR017 Correcting the bleeding phenotype in haemophilia A using lentivirally FVIII-corrected endothelial cells differentiated from hemophilic induced Pluripotent Stem Cells (iPSCs) *Cristina Olgasi, University of Piemonte, Novara*
18.15-18.30	OR018 Progenitor cells seeded collagen patches migrate and differentiate through the failing RV myocardium: which benefit on the RV function? *Virginie Lambert, INSERM U910 Aix Marseille Université , IMM Institut Mutualiste Montsouris, Paris*
16.30-18.30 *Auditorium*	**3d: Immunology/cancer immuno-gene therapy I** *Chairs: Chiara Bonini, Dirk Busch*
16.30-17.00	INV048 The tolerant immune environment of tumors governs tumor fate and efficacy of immunotherapies *David Klatzman, Sorbonne Université, UPMC Univ Paris 06, INSERM umrs959; P-HP, Hôpital Pitié-Salpêtrière*
17.00-17.30	INV049 First application of gene-edited 'universal' T cells for leukaemia *Waseem Qasim, University College London*
	Proffered papers
17.30-17.45	OR019 Modelling the cytokine release syndrome and its treatment in a long-term xenotolerant mouse model of CAR-T cell immunotherapy *Margherita Norelli, San Raffaele University, Milan*
17.45-18.00	OR020 Targeting the TCR β-constant region for specific immunotherapy of T-cell malignancies *Paul Maciocia, University College London*
18.00-18.15	OR021 Immunovirotherapy in combination with immune checkpoint inhibitors for treating glioblastoma stem cell-derived tumors *Samuel Rabkin, Massachusetts General Hospital, Harvard Medical School*
18.15-18.30	OR022 Multiple inhibitory receptors are expressed on central memory and memory stem T cells infiltrating the bone marrow of AML patients relapsing after allo-HSCT *Maddalena Noviello, San Raffaele Scientific Institute, Milan*
18:30-20:30	Poster session 1 (Odd poster numbers). See page 42 for details *Leonardo, Fra' Angelico, Paolo Uccello, Giotto and Ballatoi rooms*
20.00-23.00 *Palazzo Vecchio*	Speaker dinner (by invitation only) *Walking party departs at 20.00 from congress registration area (main entrance)*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MeiraGTx is developing innovative gene therapies for diseases including: rare inherited blindness, age-related macular degeneration (AMD), xerostomia, and neurodegenerative diseases such as amyotrophic lateral sclerosis (ALS). We are also creating novel genetic regulation platforms that promise to transform the application of gene therapy.
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PROGRAMME

THURSDAY 20 OCTOBER 2016

MAIN CONGRESS	
08.00-10.00 *Auditorium*	**4: Cancer immuno–gene therapy** *Chairs: Katherine High, Attilio Bondanza*
08.00-08.30	INV050 CAR T-cell therapy: from CD19 to other targets *Gianpietro Dotti, Department of Microbiology and Immunology, UNC Chapel Hill*
08.30-09.00	INV051 Chimeric antigen receptor T-cells - killing cancer by design *Stanley Riddell, Fred Hutchinson Cancer Research Center, University of Washington, Seattle*
09.00-09.30	INV052 Engineering T-cells for cancer therapy *Carl June, Center for Cellular Immunotherapies and Abramson Cancer Center, University of Pennsylvania, Children's Hospital of Philadelphia, Novartis Institute for Biomedical Research, Cambridge, MA*
09.30-10.00	INV053 TCR gene edited memory stem T cells for cancer immunotherapy *Chiara Bonini, Università Vita-Salute San Raffaele and Ospedale San Raffaele Scientific Institute, Milan*
10.00-10.30 *Limonaia, Passi Perduti*	Coffee break
	Parallel sessions 4a, 4b, 4c
10.30-12.30 *Auditorium*	**4a: Haematopoietic stem cells and homeostasis** *Chair: Leonard Zon, Cynthia Dunbar*
10.30-11.00	INV055 Hematopoietic stem cell attrition and regeneration in response to inflammatory stress *Michael Milsom, HI-STEM; NCT DKFZ, Heidelberg*
11.00-11.30	INV054 HSC, metabolism and fate decisions *Emmanuelle Passegué, University of California, San Francisco*
	Proffered papers
11.30-11.45	OR023 Clonal tracking of hematopoietic stem and progenitor cells *in vivo* in humans *Serena Scala, SR-Tiget, Milan*
11.45-12.00	OR024 Measles virus glycoprotein pseudotyped lentivectors allow high-level transduction of pre-stimulated and resting HSCs and correct HSCs in total bone marrow from Fanconi Anemia patients *Els Verhoeyen, CIRI; Inserm U1111, Lyon*

PROGRAMME

THURSDAY 20 OCTOBER 2016

12.00-12.15	OR025 Single-cell assay of human Hematopoietic Stem and Progenitor Cells (HSPC) following gene transfer reveals dramatic heterogeneity in HSC proliferative potential and points to a role of sphingolipids metabolic genes in HSC function *Olga Gan, University Health Network, Toronto*
12.15-12.30	OR026 Multiple allogeneic progenitors in combination function as a unit to support early transient hematopoiesis following transplantation *Makoto Otsu, Institute of Medical Science, University of Tokyo*
10.30-12.30 *Botticelli*	**4b: MSC gene and cell therapy** *Chairs: Michael Laflamme, Luis Garcia*
10.30-11.00	INV056 Use of expanded adipose stem cells in the treatment of inflammatory diseases *Wilfried Dalemans, Tigenix NV, Hasselt*
11.00-11.30	INV057 Therapeutic immune regulation by mesenchymal stromal cells *Willem Fibbe, Leiden University Medical Center*
	Proffered papers
11.30-11.45	OR027 Comprehensive characterisation of bone marrow-derived mesenchymal stromal cells from patients affected by primary immunodeficiency *Nadia Starc, Bambino Gesù Children's Hospital, Rome*
11.45-12.00	OR028 Amelioration of lung function and pulmonary tissue regeneration after treatment with Alpha-1 antitrypsin (AAT)-expressing mesenchymal stem cells (MSCs) in a murine model of elastase-induced emphysema *Sabine Geiger, Apceth GmbH & Co. KG, Munich*
12.00-12.15	OR029 Mesenchymal stromal cells prevent graft failure in a mouse model of hematopoietic stem cell gene therapy *Maria Fernandez Garcia, CIEMAT/CIBERER, IIS-FJD, UAM, Madrid*
12.15-12.30	OR030 Pulmonary artery reconstruction using cord blood-derived multipotent stem cells *in vitro* and *in vivo* study *Hiudong Jia, University of Bristol*
10.30-12.30 *Masaccio*	**4c: *In vivo* gene therapy I** *Chairs: Xavier Anguela, Hildegard Büening* Spark
10.30-11.00	INV058 Assessing and modulating immunogenicity in AAV vector mediated gene transfer *Federico Mingozzi, Genethon, Evry*
11.00-11.30	INV059 Gene therapy for haemophilia *Katherine High, Spark Therapeutics, Philadelphia, PA*

PROGRAMME

THURSDAY 20 OCTOBER 2016

	Proffered papers
11.30-11.45	OR031 Sustained expression with partial correction of neutrophil defects 5 years after intramuscular raav1 gene therapy for alpha-1 antitrypsin deficiency *Terry Flotte, University of Massachusetts Medical School, Worcester*
11.45-12.00	OR032 Liver-directed gene therapy with lentiviral vectors in animal models of haemophilia B *Michela Milani, SR-Tiget, Vita-Salute San Raffaele University, Milan*
12.00-12.15	OR033 Towards clinical gene and cell therapies for OPMD *Capucine Trollet, UPMC Univ Paris 06, UM76, INSERM U974, Institut de Myologie, CNRS FRE3617*
12.15-12.30	OR034 Red blood cells as therapeutic carrier in monogenic disorders *Giuseppa Piras, University College London*
12.30-14.00 *Limonaia, Passi Perduti*	Lunch *Posters available for viewing in Leonardo, Fra' Angelico, Paolo Uccello, Giotto and Ballatoi rooms*
	Parallel sessions 5a, 5b, 5c
14.00-16.00 *Masaccio*	**5a: Cancer stem cells** *Chairs: John Dick, Emmanuelle Passegue*
14.00-14.30	INV060 Stem cells in cancer and regenerative medicine *Michael Clarke, Stanford University, CA*
14.30-15.00	INV061 Leukemic stem cell interactions with the microenvironment: friend or foe? *Dominique Bonnet, The Francis Crick Institute, London*
15.00-15.30	INV062 Plasticity of cancer cells: lessons from gliobastomas *Inder Verma, The Salk Institute, La Jolla, CA*
	Proffered papers
15.30-15.45	OR035 microRNA-126 orchestrates a stem cell-like programme in Acute B Lymphoblastic Leukemia (B-ALL) *Carolina Caserta: SR-Tiget, Vita-Salute San Raffaele University, Milan*
15.45-16.00	OR036 A stem cell oriented phylogeny of cancers derived novel cancer gene expression signature in all undifferentiated cancers as a therapeutic target *Robert Downey, Memorial Sloan Kettering Cancer Center, New York City*
14.00-16.00 *Auditorium*	**5b: *Ex vivo* HSC-based gene and cell therapy** *Chairs: Juan Bueren, Guiliana Ferrari*
14.00-14.30	INV063 Gene therapy for primary immune deficiencies: ADA-SCID and XCGD *Don Kohn, University of California, Los Angeles, CA*

PROGRAMME

THURSDAY 20 OCTOBER 2016

14.30-15.00	INV064 Clonal expansion and long-term persistence of rhesus macaque NK cells with an adaptive phenotype as revealed by genetic barcoding *Cynthia Dunbar, National Heart, Lung, and Blood Institute, Washington DC*
	Proffered papers
15.00-15.15	OR037 Lentiviral gene therapy with busulfan conditioning for older patients with SCID-X1 *Harry Malech, National Institute of Allergy and Infectious Diseases, Bethesda, MD*
15.15-15.30	OR038 Lentiviral-mediated gene therapy in Fanconi anemia A: preclinical and first clinical studies *Susana Navarro Ordonez, CIEMAT/CIBERER, IIS-FJD, UAM, Madrid*
15.30-15.45	OR039 A clinically applicable lentiviral vector corrects NSG mice engrafting cells from patients with infantile malignant osteopetrosis *Ilana Moscatelli, Lund University*
15.45-16.00	OR040 Thalassemic bone marrow microenvironment accelerates hematopoietic stem cell ageing and exhaustion *Annamaria Aprile, SR-Tiget, Milan; University of Rome 'Tor Vergata'*
14.00-16.00 ***Botticelli***	**5c: DNA-based gene transfer and *in vivo* gene therapy II** *Chairs: Zoltan Ivics, Amber Salzman* ADVERUM BIOTECHNOLOGIES
14.00-14.30	INV065 AAV for liver directed genome editing *James Wilson, University of Pennsylvania, Philadelphia*
	Proffered papers
14.30-14.45	OR041 Diversion towards non-toxic metabolites by gene transfer for therapy of primary hyperoxaluria type *Nicola Brunetti Pierri, Tigem, Federico II University of Naples*
14.45-15.00	OR042 Development and production scale-up of an AAV8-UGT1A1 vector for the treatment of Crigler-Najjar syndrome *Fanny Collaud, Genethon, Evry,*
15.00-15.15	OR043 Improvement of gene therapy for Wilson's disease *Oihana Murillo-Sauca, Centro de Investigacion Medica Aplicada (CIMA) Pamplona*
15.15-15.30	OR044 Monitoring of anti-drug antibody responses, from development to assay validation prior to clinical trial initiation. Focus on anti-AAV responses *Sabrina Triffault, Genosafe, Evry*
15.30-15.45	OR045 Deciphering AAV vector persistence in hematopoietic progenitors *Irene Gil Farina, NCT DKFZ, Heidelberg*
15.45-16.00	OR046 Impact of vector design and administration technique in gene therapy for the treatment of age- related macular degeneration *Mehdi Gasmi, Adverum Biotechnologies Inc, Menlo Park, CA*

PROGRAMME

THURSDAY 20 OCTOBER 2016

16.00-16.30 *Limonaia, Passi Perduti*	Coffee break
16.30-18.30 *Auditorium*	**5: New technologies: targeted genome and epigenome editing, new vector design, organoids** *Chairs: Christine Mummery, George Q Daley*
16.30-17.00	INV066 Inheritable silencing of endogenous genes by hit-and-run targeted epigenetic editing *Angelo Lombardo, SR-Tiget, Milan*
17.00-17.30	INV067 Genome engineering: prospects and challenges *Feng Zhang, MIT, Cambridge, MA*
17.30-18.00	INV068 Generating a kidney from human pluripotent stem cells: where to from here? *Melissa Little, Murdoch Children's Research Institute, University of Melbourne*
18.00-18.30	INV069 From pluripotent stem cells to cortical circuits *Pierre Vanderhaeghen, Université Libre de Bruxelles*
18.30-20.00	Poster session 2 (Even poster numbers). See page 42 for details *Leonardo, Fra' Angelico, Paolo Uccello, Giotto and Ballatoi rooms*
20.30-01.00	Molecular Mingle evening – Mercato Centrale. See page 64

A unique combination

Integrated know-how, R&D, expert staff, bioprocessing and laboratory facilities and regulatory and clinical expertise

Oxford BioMedica is a world leading gene and cell therapy company focused on developing life changing treatments for serious diseases.

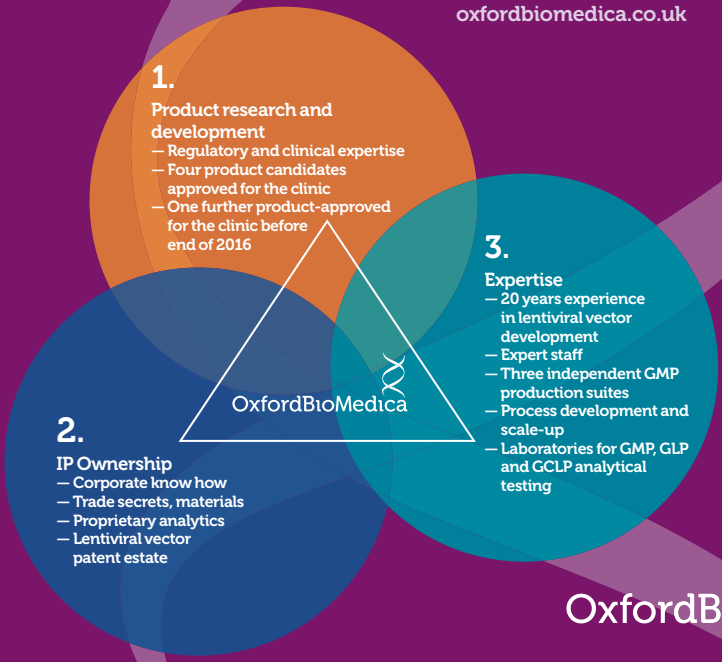
With our 20 years experience we have created a proprietary lentiviral vector gene delivery platform ("LentiVector®") which can be used for both *in vivo* and *ex vivo* products. The platform is based on our unique combination of patents and know how, expert and experienced employees, and state-of-the-art bioprocessing and laboratory facilities.

We are using the LentiVector® delivery platform to:

1. develop our own portfolio of gene and cell therapy product candidates of oncology, ophthalmology and CNS
2. help partner companies fast track and de-risk development of gene and cell therapy products to facilitate patient access to these potentially transformational therapies

The gene and cell therapy sector is now set to grow rapidly. Our "platform to product" business model is our path to generating patient benefits and sustainable shareholder value.

oxfordbiomedica.co.uk



OxfordBioMedica

Discover. Realise.

PROGRAMME

FRIDAY 21 OCTOBER 2016

	Parallel sessions 6a, 6b, 6c, 6d
09.00-10.30 *Michelangelo*	**6a: RNA-based gene transfer and integration studies** *Chairs: Eugenio Montini, Terry Flotte*
09.00-09.30	INV070 Wherever you go – there you are: tracking DNA modifications *Christof von Kalle, NCT DKFZ, Heidelberg*
09.30-10.00	INV071 Clonal tracking of engineered human hematopoiesis through integration sites analysis *Luca Biasco, SR-Tiget, Milan, Gene Therapy Programme Dana-Farber/ Boston Children's Cancer and Blood Disorders Center, University College London*
	Proffered papers
10.00-10.15	OR047 New molecular surrogate assay for genotoxicity assessment (SAGA) *Michael Rothe, Hannover Medical School*
10.15-10.30	OR048 Identification and ranking of different chromatin insulators to block vector-driven enhancer-mediated insertional mutagenesis *in vivo* *Monica Volpin, SR-Tiget, Milan*
09.00-10.30 *Auditorium*	**6b: Genome editing and gene correction** *Chairs: Thomas Barnes, Jakub Tolar*
09.00-09.30	INV072 Highly efficient gene editing in hematopoietic stem cells *Toni Cathomen, University Medical Centre, Freiburg*
09.30-10.00	INV073 Genome editing for Duchenne muscular dystrophy *Charles Gersbach, Duke University, USA*
	Proffered papers
10.00-10.15	OR049 Targeted gene therapy in the treatment of X-Linked Hyper-IgM Syndrome *Caroline Kuo, University of California Los Angeles (UCLA)*
10.15-10.30	OR050 Identification of high-fidelity Cas9 variants using a yeast-based screening *Antonio Casini, University of Trento*
09.00-10.30 *Masaccio*	**6c: Cancer gene therapy** *Chairs: Len Seymour, Inder Verma*
09.00-09.30	INV074 Cancer virotherapy with oncolytic adenoviruses *Ramon Alemany, Catalan Institute of Oncology, Barcelona*
09.30-10.00	INV075 Development of prostate cancer gene therapy in Japan *Yasutomo Nasu, Okayama University*

PROGRAMME

FRIDAY 21 OCTOBER 2016

	Proffered papers
10.00-10.15	OR051 Clinical update, molecular analyses, and proposed mechanism of action of Toca 511 a retroviral replicating vector in three ascending dose trials in patients with recurrent high-grade glioma *Doug Jolly, Tocagen Inc, San Diego, CA*
10.15-10.30	OR052 Anti-tumor potency of cancer vaccine ONCOS-102 in the treatment of malignant mesothelioma in preclinical and clinical studies *Lukasz Kuryk, Targovax Oy, Helsinki*
09.00-10.30 ***Botticelli***	**6d: Immunology and allergy** ***Chairs: Federico Mingozzi, Ron Crystal***
09.00-09.30	INV076 Gene therapy for hereditary and acquired life-threatening, immune-mediated disorders *Ron Crystal, Weill Cornell Medical College, New York City*
09.30-10.00	INV077 Gene therapy-based approach for immune tolerance induction *Maria Grazia Roncarolo, Stanford School of Medicine, CA*
	Proffered papers
10.00-10.15	OR053 Intrinsic defect in Was-/- platelets: studies in conditional mouse model and WAS gene therapy treated patients *Lucia Sereni, SR-Tiget, Milan*
10.15-10.30	OR054 Gene therapy for Ebola virus infections based on AAV vectors and Zmapp antibody cocktail *Bruno Gaillet, Université Laval, Quebec*
10.30-11.00 *Limonaia, Passi Perduti*	Coffee break
11.00-12.00 ***Auditorium***	**6: Gene therapy in the market** ***Chairs: Luigi Naldini, Sven Kili***
11.00-11.15	INV078 Primary immune deficiencies: a natural target for *ex vivo* gene therapy *Jonathan Appleby, GSK, Brentford*
11.15-11.30	INV079 *Ex vivo* gene therapy in ADA-SCID: clinical data and experiences to date *Alessandro Aiuti, SR-Tiget, Milan*
11.30-11.45	INV080 Translating experimental gene therapy into clinical reality *Sol Ruiz, AEMPS, Madrid*
11.45-12.00	INV081 Patient management through the gene therapy process *Julie Venners-Christensen, GSK, Brentford*

PROGRAMME

FRIDAY 21 OCTOBER 2016

12.00-13.00 *Auditorium*	**7: *In vivo* gene therapy** *Chairs: Olivier Danos, Amit Nathwani*
12.00-12.30	INV082 Gene therapy of mucopolysaccharidosis VI *Alberto Auricchio, Tigem, Naples*
12.30-13.00	INV083 Making a good vector even better: novel rAAVs for classical gene therapy and genome editing *Mark Kay, Stanford University, CA*
13.00-14.00 *Limonaia, Passi Perduti*	Lunch
	Parallel sessions 7a, 7b, 7c, 7d
14.00-15.30 *Auditorium*	**7a: Immunology/cancer immuno-gene therapy II** *Chairs: Pierre Cordelier, Ramon Alemany*
14.00-14.30	INV084 T-Cell and cancer immunotherapy *Dirk Busch, Technische Universität München*
14.30-15.00	INV085 Macrophage-based delivery of immunostimulatory and antiangiogenic molecules into the tumor microenvironment *Bernhard Gentner, SR-Tiget, Milan*
	Proffered papers
15.00-15.15	OR055 Reduced CAR tonic signaling and methods to enhance memory T cells result in improved *in vivo* efficacy in human multiple myeloma xenograft models *Richard Morgan, bluebirdbio*
15.15-15.30	OR056 Balance of Anti-CD123 Chimeric Antigen Receptor (CAR) binding affinity and density in an *in vitro* model of acute myeloid leukemia *Sarah Tettamanti, Universita' Milano Bicocca, Osp. San Gerardo/Fondazione MBBM, Monza*
14.00-15.30 *Botticelli*	**7b: Gene silencing: from small non-coding RNAs to epigenetic editing and gene disruption** *Chairs: Tony Cathomen, Angelo Lombardo*
14.00-14.30	INV086 ZFN-mediated genome editing in the liver – towards the correction of lysosomal storage diseases *Michael Holmes, Sangamo BioSciences Inc, Richmond, CA*
14.30-15.00	INV087 MicroRNA-based therapeutics in cancer *Frank Slack, Harvard Medical School, Cambridge, MA*

PROGRAMME

FRIDAY 21 OCTOBER 2016

	Proffered papers
15.00-15.15	OR057 Transcriptional silencing via synthetic DNA binding protein lacking canonical repressor domains as a potent tool to generate therapeutics *Salvatore Botta, Tigem, Naples*
15.15-15.30	OR058 ASO-mediated Dnm2 knockdown prevents and reverts Myotubular myopathy *in vivo* in mice *Belinda Cowling, IGBMC, Strasbourg*
14.00-15.30 *Michelangelo*	**7c: Manufacturing of cell and gene therapy products** *Chairs: Gabor Veres, Otto Merten*
14.00-14.30	INV088 Challenges in vector and cell manufacturing in gene therapy *Paolo Rizzardi , MolMed S.p.A, Milan*
14.30-15.00	INV089 Mastering the challenges of manufacturing: the critical roles of closed systems and automation *Ian Johnston, Miltenyi Biotec, Bergisch Gladbach*
	Proffered papers
15.00-15.15	OR059 Improving the purity of Adeno-associated viral vector preparations using DNA minicircle technology *Hildegard Büning, University of Cologne, DZIF, University Hospital Cologne, Hannover Medical School*
15.15-15.30	OR060 Staurosporine Increases Lentiviral Transduction of Human CD34+ Cells *Melissa Bonner, bluebirdbio, Cambridge, MA*
14.00-15.30 *Masaccio*	**7d: CNS gene therapy** *Chairs: Alessandra Biffi, Nicole Deglon*
14.00-14.30	INV090 AAV-CYP46A1 brain administration restores cholesterol metabolism and is neuroprotective in Huntington's disease *Nathalie Cartier-Lacave, INSERM UMR1169, Université Paris-Sud; CEA, DSV, FBM, MIRCen, Fontenay-aux-Roses*
14.30-15.00	INV091 AVXS-101 phase 1 gene therapy clinical trial in SMA type 1 *Jerry Mendell, Nationwide Children's Hospital, Ohio State University*
15.00-15.15	*Proffered papers* OR061 From bench to bedside: A novel approach in the treatment of SMA Type 1 with gene therapy *Brian Kaspar, Nationwide Children's Hospital, The Ohio State University Medical Center, AveXis, Inc*
15.15-15.30	OR062 Improvements in motor function and PET findings following gene transfer to the patients with AADC deficiency *Taka Yamagata, Jichi Medical University*

PROGRAMME

FRIDAY 21 OCTOBER 2016

15.30-15.50 *Limonaia, Passi Perduti*	Coffee break
15.50-17.45 *Auditorium*	**ESGCT AGM and presidential symposium and awards ceremony** *Chairs: Nathalie Cartier-Lacave, Luigi Naldini*
15:50-16:15	ESGCT AGM
16.15-17.00	INV092 Milestones and barriers in hematopoietic stem cell derivation from pluripotent stem cells *George Q Daley, Boston Children's Hospital, MA*
17.00-17.30	**Outstanding Achievement Award** INV093 Progress for gene therapy in haemophilia *Amit Nathwani, University College London*
17.30-17.45	**Young Investigator Awards** OR063 Towards clinical translation of gene editing technologies for empowering adoptive immunotherapy or correcting inherited mutations *Pietro Genovese, SR-Tiget, Milan*
17.45-19.00 *Auditorium*	**Germline editing debate** *Chairs: Roberto Buccione* *Annelien Bredenoord, University Medical Center Utrecht* *Giuseppe Testa, European Institute of Oncology, University of Milan* *George Q Daley, Boston Children's Hospital, MA* *Nathalie Cartier-Lacave, INSERM UMR1169, Université Paris-Sud; CEA, DSV, FBM, MIRCen, Fontenay-aux-Roses* *Luigi Naldini, SR-Tiget, Milan* *Feng Zhang, The Broad Institute, MIT, Cambridge, MA*
19.00-20.00	Closing drinks

VISITING FLORENCE

It is estimated that 40% of the world's most important artworks are found in Italy, and 30% of these are located in Florence. Situated in the heart of Italy, surrounded by the wine-growing hills of Chianti, the city enchants visitors with its timeless charm.

The names Strozzi, Rucellai and Pitti can be found all over Florence, but it was the Medici family, who led the city for over 300 years, that nurtured the greatest flowering of Renaissance art. The paintings of Botticelli, the sculptures of Michelangelo, and the rusticated palaces of Michelozzo all flourished under their rule.

The mix of ancient and modern culture makes Florence an enchanting and inspiring city. Florence is also a busy cosmpolitan centre, offering arts festivals, historic cafes, excellent restaurants and picturesque 'trattorie' serving the best regional Italian cuisine.

Don't miss the Cathedral with its splendid dome designed by Brunelleschi; Giotto's Campanile; and the Uffizi Gallery. But it is the hidden and lesser known Florence that will remain in your heart: the small churches, street markets and traditional 'trattorias', and the tiny artisan workshops which hand down ancient techniques from generation to generation.

Shopping

Florence offers a vast array of shops, from famous designers' boutiques to vintage and hand crafted artisan stalls. Look for luxury items in Via Tornabuoni and Via della Vigna; leather goods in Limonaia, Passi Perduti Santa Croce and Limonaia, Passi Perduti San Lorenzo; antiques in Via Maggio and Via de' Fossi, and jewellery on Ponte Vecchio.

Shops open from 10am–1pm and 3.30–7.30pm daily. In the main shopping area you will find shops open all day.

Tipping

There is no tipping in Italy. Customers pay the exact amount that appears on the bill.

VAT

Italy's VAT for business transactions and purchases is 22 percent, and the tax for basic products 4 to 10 percent. The VAT is only paid by European Union consumers. All published prices (including restaurant menus, taxi fares and supermarket prices) include consumer tax.

TOURS

Tours are organised by MCO. All information is available from their booth next to the main registration desk. Spouses and family members are welcome on all tours. 'Highlights of Florence' will take place every day at various times; all other tours will take place once. Tours in Florence require a minimum of 10 participants; tours around Florence require a minimum of 15 participants.

Highlights of Florence

This tour takes in the Ponte Vecchio, the bridge lined with jewellery shops since 1552 when Duke Cosimo I issued an edict for jewelers to replace all of the butchers shops previously lining the landmark. We then pass the Uffizi Gallery to see the fake David where Michelangelo's real statue once stood. Next we will visit Limonaia, Passi Perduti della Signoria and the seat of the Florentine city government. We'll do a quick stop at the church of Orsanmichele, followed by Florence's formidable landmark, the Duomo, famous for Brunelleschi's dome, Giotto's colorful bell tower and the beautiful Baptistry.

18 Oct at 10am; 19 Oct at 3pm; 20 Oct at 3pm
21 Oct at 10am; Duration: 3 hours
Bookings: www.esgct-isscr2016.com/tours

Inferno Tour

Fans of Dan Brown's novel 'Inferno' can follow in the footsteps of Robert Langdon, discover Dante and decipher the codes and mysteries in the American writer's latest novel. Learn about Dante's life and how he changed Florence and Italy, leaving a lasting impact upon everything from the Italian language and master artworks to the Catholic church itself with one epic poem – his 'Divine Comedy'.

We reveal insider insights provided by the only living being referred to in the book. Visit one of the most iconic buildings of Florence, the Town Hall or Palazzo Vecchio where the movie was recently filmed. We'll walk to the Pitti Palace and to the Baptistry too. You'll see Florence as never before!

21 Oct at 3pm; Duration: 3 hours
Bookings: www.esgct-isscr2016.com/tours

Italian cities

There are also full day tours of the picturesque cities of Siena, Lucca and Pisa, and San Gimignano and Chianti.
For more information:
www.esgct-isscr2016.com/tours

EUROPEAN SOCIETY OF GENE AND CELL THERAPY ACHIEVEMENT AWARDS

Outstanding Achievement Award: ESGCT presents one award for an established researcher who has made a long-term, outstanding contribution to the field: €2,000 honorarium and 30-minute presentation during the annual congress.

Young Investigator Awards: €1,000 honorarium and a 15-minute presentation during the annual congress for researchers who are showing exceptional promise.

Travel grants: Supported by the national societies, up to 10 awards of €300 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.aspx**.

Note: Eligibility criteria apply

ESGCT/ISSCR EVALUATION AND CERTIFICATE OF ATTENDANCE

We do hope you have enjoyed the ESGCT/ISSCR/ABCD Collaborative Congress 2016. 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 the online questionnaire.

You will be sent an email with the link and information for the survey during or shortly after the congress. Once you have completed the survey, you will receive your Certificate of Attendance by email within the following 24 hours.

ESGCT and ISSCR Teams

LYSOGENE

On the cutting edge of CNS gene therapy science

Lysogene is proud to support the ESGCT and its members

Lysogene is a global biotechnology company, a leader in the basic research and clinical development of gene therapy for neurodegenerative disorders. **Lysogene**'s mission is to radically improve the health of patients suffering from incurable life threatening conditions by developing AAV vectors that have demonstrated their effectiveness in safely delivering genetic material to the central nervous system.

Lysogene's most advanced product candidate is rAAV vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase (hSGSH) for the treatment of mucopolysaccharidosis IIIA (MPS IIIA). The recently completed phase I/II study in four MPS IIIA children demonstrated that the gene therapy and neurosurgical procedure is safe, well tolerated and exploratory efficacy profiles are encouraging (Tardieu 2014). A multinational phase IIb pivotal clinical trial is under preparation with a second generation gene therapy.

Lysogene also has a program underway for the development of a rAAVrh.10 carrying the human beta-galactosidase (βgal) for the treatment of GM1 gangliosidosis.

Lysogene is currently expanding its pipeline to other genetic diseases affecting the central nervous system.

18-20, rue Jacques Dulud
92200 Neuilly-sur-Seine - France

245 First Street, 18th Floor
Cambridge MA 02142 - United States

www.lysogene.com

AFMTÉLÉTHON

CURE THROUGH INNOVATION

2 AND 3 DECEMBER 2016

AFM-Telethon federates patients who are affected by neuromuscular diseases and their families. In order to fight those diseases, AFM-Telethon chose to initiate innovative actions and a strategy of general interest that benefits all rare diseases and all persons with disabilities.

Thanks to donations from the French annual Telethon, AFM-Telethon has become a major player in biomedical research for rare diseases in France and worldwide. While the number of human trials is on the increase, the Association is more than ever focused on its objectives: therapeutic efficacy and access to drugs for patients at a fair and controlled price.

AFM-TELETHON IS:

➔ **4 leading laboratories** in innovative biotherapies grouped within the Biotherapy Institute for Rare Diseases: **Genethon** and **Atlantic Gene Therapies** for gene therapy of rare diseases, **Institute of Myology** for research and treatments of neuromuscular disorders, **I-Stem** for stem cell therapy of genetic diseases.

➔ Funding for **285 research programmes** and young researchers in 2015;

➔ Support for **37 current and upcoming clinical trials** for 27 rare disorders of vision, muscles, brain, heart, skin, liver, blood...;

➔ A **platform for paediatric clinical trials** for neuromuscular disorders, I-Motion;

➔ A **centre for pre-industrial production of gene therapy products**, Genethon Bioprod, and soon, an industrial platform for the development and production of gene and cell therapies.

For more information:
www.afm-telethon.com

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Special Joint Issue on Stem Cells and Gene Therapy

Guest Editor: **Luigi Naldini**

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GENETHON
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GENE THERAPY FOR RARE DISEASES

Advancing Therapies From Research to Patient Treatment

Genethon, created by AFM-Telethon, has the mission to make innovative gene therapy treatments available to patients affected with rare genetic diseases. Having played a pioneering role in deciphering the human genome, Genethon is today one of the leading organizations for the development of gene therapy treatments.

The pipeline of Genethon includes products currently in clinical trials and at preclinical stages, for muscular dystrophies , immune deficiencies, blood, ocular and liver diseases.

These products are developed either with Genethon as sponsor, or in partnership with private companies and academic institutions.

FOR MORE INFORMATION and details:
www.genethon.fr

Member of
BIOTHERAPIES INSTITUTE
CURE THROUGH INNOVATION

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The European Society of Gene and Cell Therapy has, as its objective, the promotion of science and research.

We achieve this in part through scientific and educational activities, in particular through measures aimed at the promotion and the exchange of information and ideas with regard to gene therapy, cell therapy, genetic vaccination, the encouragement of research fields, and clinical applications.

As such we would like to support the activities of national societies that share this goal – please see below for information and contact details of some national societies to help you, should you wish to get in touch with any of them.

United Kingdom

www.bsgct.org

office@bsgct.org.uk

Next meeting:
British Society for Gene and Cell Therapy Annual Conference
and UK Regenerative Medicine Platform Joint Meeting
Royal Welsh Academy of Music and Drama, Cardiff, Wales
19–20 April 2017

Germany

www.dg-gt.de

hildegard.buening@uk-koeln.de

Next meeting:
ESGCT Collaborative Congress with the DG-GT
Berlin, Germany
17–20 October 2017

Finland

www.fsgt.fi

France

www.sftcg.fr

office@sftcg.fr

Next meeting:
ESGCT Collaborative Congress with the SFTCG
Lausanne, Switzerland 2018

The Netherlands

www.nvgct.nl

p.j.bosma@amc.uva.nl

Next meeting:
NVGCT Spring Symposium
Congrescentrum De Werelt, Lunteren
16–17 March 2017

Spain

www.setgyc.es

office@setgyc.es

Next meetings:
Gene and Cell Therapy Spring School, 5–7 April 2017, Granada, Spain

Spanish Society for Gene and Cell Therapy Biennial Congress
14–16 March 2018, Palma de Mallorca

îledeFrance

DIM BIOTHERAPIES

The **DIM Biotherapie** - Domaine d'Intérêt Majeur Biotherapie - is a scientific network sponsored by the **Paris Ile de France Region** to support, develop and structure a collaborative research network in the field of Regenerative Medicine. Gene Therapy, Cell therapy, Stem Cell Research, Developmental Biology, and Transplantation. Through annual call-for-proposals, the Dim Biotherapy finances doctoral contract salaries (open to European students), laboratory small and large equipment and scientific workshops in The Paris Ile de France Region.

A 3 days interactive workshop **'Biotherapies for Genetic Diseases at Université Paris Saclay : Programs and Perspectives'** will take place in the wonderful city of Versailles in **April 2017** (*date to be confirmed/free registration*). The **BiotherAlliance Network of the Paris Saclay University** will be presented at this workshop.

BiotherAlliance
université
PARIS-SACLAY

EUROPEAN SOCIETY OF GENE & CELL THERAPY

GENE AND CELL THERAPY
SPRING SCHOOL

5–7 APRIL 2017

Granada, Spain

Don't miss this unique opportunity to participate in an intensive three day training course with leading researchers from the fields of Gene and Cell Therapy. Debates and networking with Europe's current and future leaders in the field.

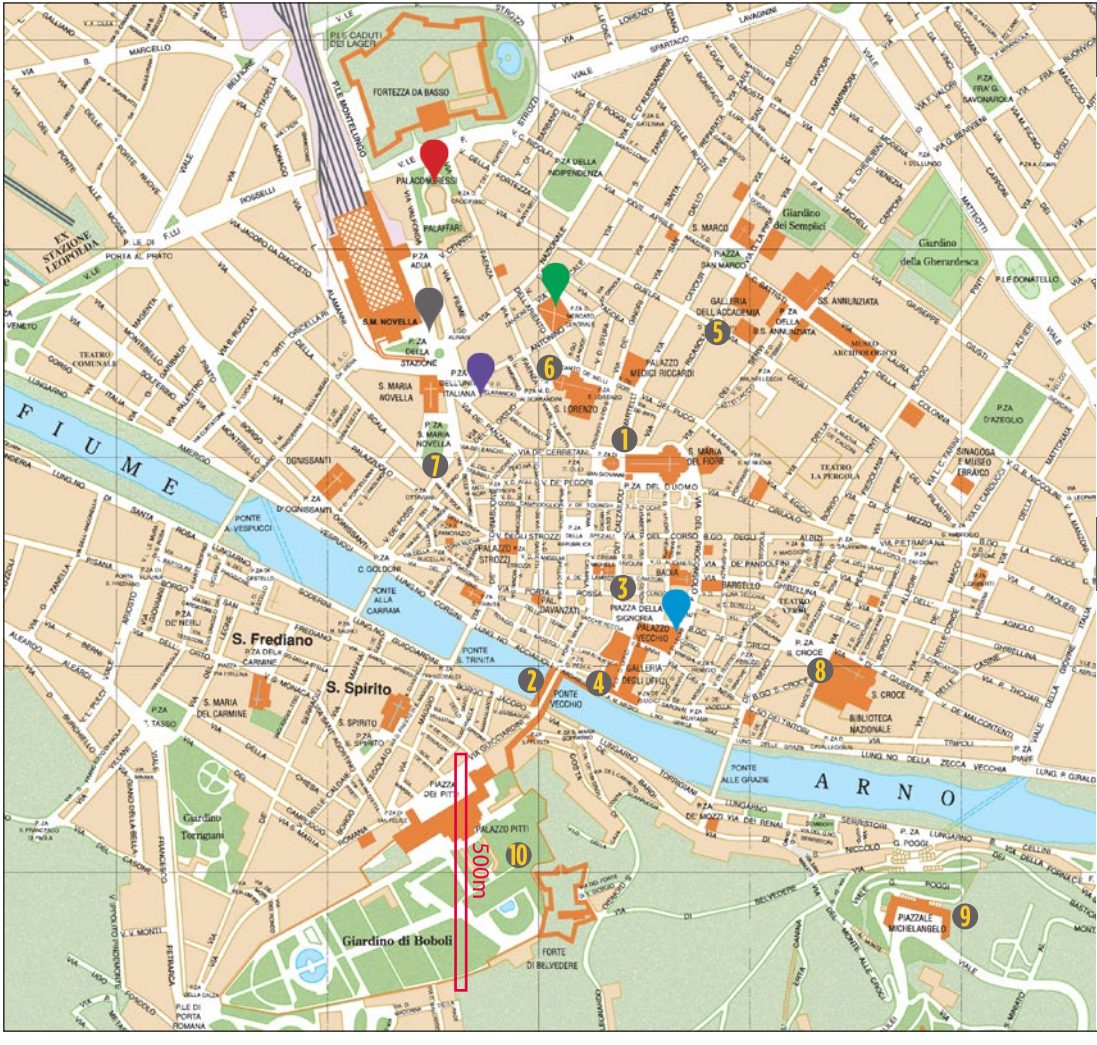
Speakers:

Ramon Alemany, Robin Ali, Gloria González Aseguinolaza, Fatima Bosh, Hildegard Büening, Juan Bueren, Nathalie Cartier-Lacave, Guillermo Guenechea Zoltan Ivics, Ander Izeta, José Luís Labandeira, Paco Martin, Manuel Ramírez Orellana, Juan Carlos Ramirez, Angel Raya, Paula Río, Pilar Sepúlveda, Adrian Thrasher, Juan José Toledo

Themes:

Understanding the social impact of gene and cell therapy; gene and cell therapy tools; cellular plasticity and reprogramming; blood disorders; neurodegenerative diseases; cardiovascular diseases; cancer; metabolic and lysosomal storage diseases; muscular and skin diseases; new frontiers in gene and cell therapy

Contact office@esgct.eu for details



MEETING SPOTS

GRAND HOTEL BAGLIONI
Piazza dell'Unità Italiana, 6
SPEAKER HOTEL

PALAZZO DEI CONGRESSI
Piazza Adua, 1
CONGRESS VENUE

MERCATO CENTRALE
Piazza del Marcato Centrale
CONGRESS EVENING 20/10/2016

PALAZZO VECCHIO
Piazza della Signoria
SPEAKER DINNER 19/10/2016

SANTA MARIA NOVELLA
Piazza della Stazione
TRAIN STATION

FLORENCE HIGHLIGHTS

1. DUOMO AND BAPTISTERY
2. PONTE VECCHIO
3. PIAZZA DELLA SIGNORIA
4. UFFIZI MUSEUM
5. GALLERIA DELL'ACCADEMIA MUSEUM
6. CAPPELLE MEDICEE MUSEUM
7. SANTA MARIA NOVELLA SQUARE
8. SANTA CROCE SQUARE
9. PIAZZALE MICHELANGELO
10. BOBOLI GARDENS

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

EUROPEAN SOCIETY OF
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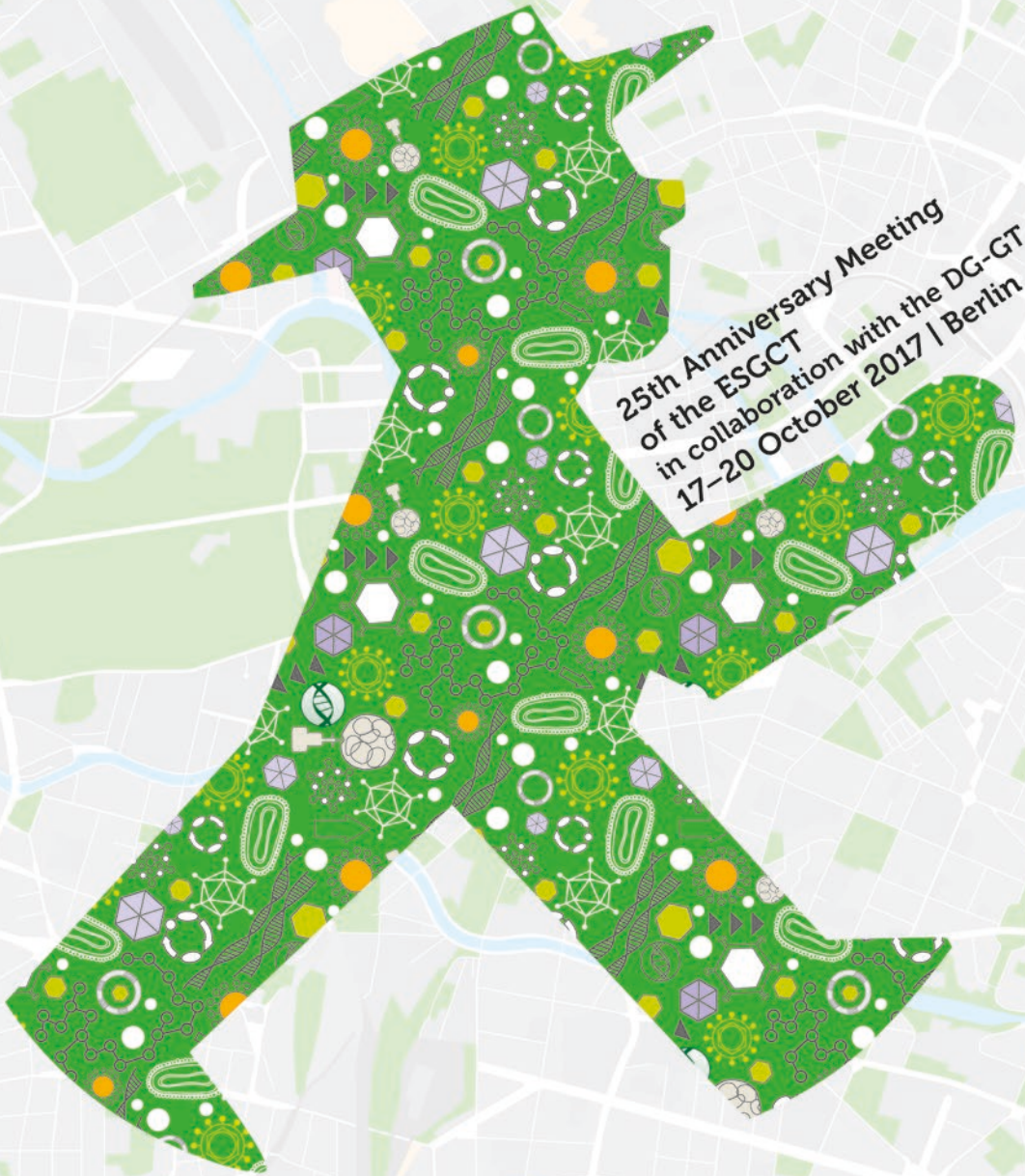

25th Anniversary Meeting of the ESGCT in collaboration with the DG-GT

17–20 October 2017 | Berlin

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