Welcome to...

FLORENCE

Palazzo dei Congressi e Palazzo degli Affari
Limonaia, Passi Perduti 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

Speaker ready room: Vasari
Press room: Filippo Lippi
Registration & 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 08.30–14.30

Tourism 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): +39 892.021
Tourist Information Center in Via Cavour 1/r;
+39 055 290832
Tourist Information Center in Limonaia, Passi
Perduti 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
Limonaia, Passi Perduti dell’Unità Italiana, 6
Tel: +39 055 23580
Fax: +39 055 2358895
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.
We could not run this meeting without the help of all our partners. Thank you!
We are delighted to present an exceptional field of award winners in 2016

Outstanding Achievement

Amit Nathwani, University College London

Progress for gene therapy in haemophilia

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 Mazara, San Raffaele Scientific Institute, Milan
Margherita Norelli, San Raffaele University, Milan
Yein Nam, University of Manchester
Matthew Elitt, Case Western Reserve University, Cleveland, OH
Lucia Sereni, SR-Tiget, IRCCS San Raffaele Scientific Institute, Milan

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

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 our first-rate scientific programme featuring:

- 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 the beautiful historical downtown of Florence, where you will find a high concentration 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 center 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 cansacenza”, 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 context, 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, don’t forget to 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 meeting and are here to help run it 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
ESGCT BOARD

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Leonard I Zon, Boston Children’s Hospital, Harvard Medical School
Nancy Witty, Chief Executive Officer ISSCR, Chicago, Illinois
CONGRESS VENUE

PALAZZO AFFARI
Lower ground floor
Michelangelo (parallel sessions)

Ground floor
Botticelli (parallel sessions)

First floor
Piero Della Francesca (parallel sessions)
Masaccio (meeting room)

Second floor
Fra Angelico (posters)
Leonardo (posters)

Donatello (meeting room)

Fourth floor
Uccello, Giotto (meeting rooms)

LIMONAIA
Exhibition and catering
Cloakroom and left luggage

PALAZZO DEI CONGRESSI
-1
Brunelleschi Auditorium (plenary room)
Passi Perduti (exhibition hall and catering)

Lower Ground
Hugo Club & Salone (exhibition hall and catering)

Ground Floor
Vasari (speaker room)
Tiziano (unmanned cloakroom)
Filippo Lippi (press room)
Caravagio, Raphael (meeting rooms)
The first licensed autologous ex vivo 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 Italian SPC can be found at: https://www.edott.it/GskInforma/Prodotto/strimvelis.aspx

Adverse events should be reported. Reporting forms and information can be found at http://www.mhra.gov.uk/yellowcard.

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

References

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
GETTING SOCIAL WITH ESGCT & ISSCR

Follow our official channels on social media:

www.facebook.com/ESGCT and www.facebook.com/ISSCR
@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. 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!

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!

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 https://uquiz.com/OLnLt

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!

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.

Programme at a Glance

Tuesday 18 October 2016

Clinical Trial and Commercialisation Workshop

Fourth Floor (Uccello Room)

08.00-09.00 Registration
09.00-09.20 Planning a clinical trial
09.20-10.00 Manufacturing of gene and cell products
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
13.00-14.00 Lunch (Fourth floor Giotto room)

Education Day

Morning session: -1 Floor Michelangelo
Afternoon session: Brunelleschi Auditorium
Sponsors: Supersist; Dimension Therapeutics

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 – Passi Perduti
13.30-14.30 E4a: Immunotherapy & transdifferentiation
14.30-15.30 E4b: Gene editing
15.30-16.00 Coffee break
**PUBLIC ENGAGEMENT DAY FOR PATIENT ASSOCIATIONS AND SCIENCE DIALOGUES WITH CITIZENS:**
Leading edge therapies for rare diseases
First floor (Piero Della Francesca Room)

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
<tbody>
<tr>
<td>09.00-09.45</td>
<td>Registration</td>
</tr>
<tr>
<td>09.45-10.15</td>
<td>Gene therapy</td>
</tr>
<tr>
<td>10.15-10.45</td>
<td>How to foster access to therapies</td>
</tr>
<tr>
<td>10.45-11.15</td>
<td>Safety studies</td>
</tr>
<tr>
<td>11.15-11.45</td>
<td>Science and bio-ethics</td>
</tr>
<tr>
<td>11.45-13.00</td>
<td>Discussion</td>
</tr>
<tr>
<td>13.00-14.00</td>
<td>Lunch &amp; networking (in the room)</td>
</tr>
<tr>
<td>14.00-14.30</td>
<td>New frontiers in science</td>
</tr>
<tr>
<td>14.30-16.30</td>
<td>Role playing: science dialogues</td>
</tr>
<tr>
<td>16.30-17.00</td>
<td>Closing remarks</td>
</tr>
</tbody>
</table>

**MOLMED SYMPOSIUM**
An entrepreneurial approach to translate academic knowledge into therapeutic solutions for all patients
Ground Floor (Botticelli Room)

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
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</thead>
<tbody>
<tr>
<td>12.30-14.00</td>
<td>Lunch and registration (in the room)</td>
</tr>
<tr>
<td>14.00-14.15</td>
<td>When pioneers in cell &amp; gene therapy come up with a ‘business’ idea</td>
</tr>
<tr>
<td>14.15-14.30</td>
<td>Academia ready to be a productive partner for biotech companies</td>
</tr>
<tr>
<td>14.30-14.45</td>
<td>How the financial market operated and operates in sustaining the biotech sector development</td>
</tr>
<tr>
<td>14.45-15.00</td>
<td>A picture of the European biotech sector: strengths and weaknesses</td>
</tr>
<tr>
<td>15.00-15.30</td>
<td>Round table</td>
</tr>
</tbody>
</table>

**MAIN CONGRESS**

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
<tbody>
<tr>
<td>16.00-17.00</td>
<td>ESGCT / ISSCR 2016 Opening: welcome and introduction</td>
</tr>
<tr>
<td></td>
<td>Auditorium</td>
</tr>
<tr>
<td></td>
<td>Sponsor: bluebird bio</td>
</tr>
<tr>
<td>17.00-19.00</td>
<td>1: Neural diseases: modelling, reprogramming and transplantation in brain and retina</td>
</tr>
<tr>
<td></td>
<td>Auditorium</td>
</tr>
<tr>
<td></td>
<td>Sponsor: RegenXbio</td>
</tr>
<tr>
<td>19.00-20.00</td>
<td>Welcome reception</td>
</tr>
<tr>
<td></td>
<td>Limonaia, Passi Perduti</td>
</tr>
<tr>
<td>19.00-21.00</td>
<td>Molecular therapy ‘meet the editor’ reception</td>
</tr>
<tr>
<td></td>
<td>Sponsor: Molecular Therapy</td>
</tr>
</tbody>
</table>
### PROGRAMME AT A GLANCE

#### WEDNESDAY 19 OCTOBER 2016

| 08.30-10.30 | 2: Hematopoietic stem cells: from biology to clinical applications  
|             | Auditorium  
|             | Sponsor: Genethon |
| 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 |
| 12.30-14.00 | Lunch – Limonaia, Passi Perduti  
|             | Odd numbered posters available for viewing in Leonardo & Fra Angelico rooms |
| 12.45-13.45 | Lunch Symposium: Regulatory workshop for ATMPs  
|             | Michelangelo  
|             | Sponsor: BoReliance |
| 14.00-15.30 | 3: Skeletal and cardiac muscle stem cells: from biology and reprogramming to clinical applications  
|             | Auditorium  
|             | Sponsor: FinVector |
| 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 |
| 18.30-20.30 | Poster session 1 (Odd poster numbers). See page 42 for details  
|             | Leonardo & Fra Angelico |
| 20.00-23.00 | Speaker dinner (by invitation only)  
|             | Palazzo Vecchio |

#### THURSDAY 20 OCTOBER 2016

| 08.00-10.00 | 4: Cancer immuno–gene therapy  
|             | Auditorium  
|             | Sponsors: Oxford BioMedica |
| 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  
|             | Masaccio  
|             | Sponsors: Spark Therapeutics |
| 12.30-14.00 | Lunch – Limonaia, Passi Perduti  
|             | Even numbered posters available for viewing in Leonardo & Fra Angelico rooms |
| 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 II  
|             | Botticelli  
|             | Sponsor: Adverum |
| 16.00-16.30 | Coffee break – Limonaia, Passi Perduti |
| 16.45-18.45 | 5: New technologies: targeted genome and epigenome editing, new vector design, organoids  
|             | Auditorium  
|             | Sponsor: Editas Medicine |
| 18.30-20.00 | Poster session 2 (Even poster numbers) See page 44 for details |
| 20.30-01.00 | Molecular Mingle evening – Mercato Centrale. See page 66 |
# Programme at a Glance

**Friday 21 October 2016**

## Main Congress

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Location</th>
<th>Sponsor</th>
</tr>
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</table>
| 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                                                                                 |               |                       |
| 10.30-11.00  | Coffee break – Limonaia, Passi Perduti                                                      |               |                       |
| 11.00-12.00  | 6: Gene therapy in the market  
              Auditorium  
              Sponsor: GSK                                                                                 |               |                       |
| 12.00-13.00  | 7: In vivo gene therapy  
              Auditorium  
              Sponsor: Biogen                                                                             |               |                       |
| 13.00-14.00  | Lunch – Limonaia, Passi Perduti                                                            |               |                       |
| 14.00-15.30  | 7a: Immunology/cancer immuno-gene therapy II  
              Auditorium                                                                                 |               |                       |
|              | 7b: Gene silencing for small non-coding RNA’s to epigenetic editing and gene disruption  
              Boticell  
              Sponsor: Sangamo BioSciences                                                                  |               |                       |
|              | 7c: Manufacturing of cell and gene therapy products  
              Michelangelo  
              Sponsor: Molmed                                                                             |               |                       |
|              | 7d: CNS gene therapy  
              Masaccio  
              Sponsor: Avexis                                                                               |               |                       |
| 15.30-15.50  | Coffee break – Limonaia, Passi Perduti                                                      |               |                       |
| 15.50-17.45  | Presidential symposium and awards ceremony  
              Auditorium  
              Sponsor: Bayer                                                                                |               |                       |
| 15.50-16.15  | ESGCT AGM                                                                                   |               |                       |
| 17.45-19.00  | Germline editing debate                                                                     |               |                       |
| 19.00-20.00  | Closing drinks                                                                              |               |                       |
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
Oncolyis
Gene editing
Cardiovascular, muscle and pulmonary gene and cell therapy
Vector development
Regenerative therapies
Metabolic and lysosomal storage diseases
Cancer predisposition, ageing and genetic instability syndromes
Blood disorders
Cancer gene therapy

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

Small- and medium-size enterprises (SMEs) in the field can apply, even as 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:
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
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- Process innovation and development
- cGMP viral vector production
- Cell banking and analytical services

WE OPEN A NEW WAY FOR TREATING NEURODEGENERATIVE DISEASES

BrainVectis is committed to developing gene therapy programs to treat neurodegenerative disorders, first Huntington’s disease and Alzheimer’s disease. We target brain cholesterol metabolism, which is impaired in neurodegenerative conditions.

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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

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 1000 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: 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 & 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 pre-clinical, 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
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.

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

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

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.
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

Merk, 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, 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

REGENX BIO is a leading biotechnology company focused on the development, commercialization 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
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 services 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
- 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 pre-clinical phase, through clinical trials and to the market. Come and visit us at booth 28–29.

www.finvector.com

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

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

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
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

GOLD PARTNERS

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www.oncorus.com

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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
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 pre-clinical, clinical and registration programmes. Chiesi employs over 4,500 people, 560 of whom are solely dedicated to Research and Development activities. www.chiesigroup.com

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 product. 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 specialised 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, HIV, 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
Orchard Therapeutics is a clinical-stage biotechnology company with operations in London and the United States and 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

Pfizer Inc: Working together for a healthier world®. At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world’s best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world’s premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. For more information, please visit us at www.pfizer.com. In addition, to learn more, follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

www.pfizer.com

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

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
BRONZE PARTNERS

**apceth – The cell engineering company!**

**PROPRIETARY TECHNOLOGY & PRODUCTS**
- Clinical stage biopharmaceutical company
- Leader in the development of genetically engineered cell therapies
- Native and engineered (2nd generation) mesenchymal stem cells
- Cancer, immunomodulation and tissue regeneration

**CONTRACT DEVELOPMENT & MANUFACTURING ORGANIZATION**
- Reliable and high-performance partner
- Complex cell-based and gene therapy products (ATMPs)
- Product and process development, GMP manufacturing
- GMP-certified since 2010

[www.apceth.com](http://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](http://www.genenta.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. Cx601 has completed a Phase I sepsis challenge trial and a Phase 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](http://www.tigenix.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](http://www.sanofigenzyme.com)
**POSTER SESSION 1**

**WEDNESDAY 19 OCTOBER 2016, 18.30-20.30**

2nd floor, Fra Angelico & Leonardo Rooms. For location of posters see opposite page.

**POSTERS BY CATEGORY (ODD NUMBERS)**

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# POSTER SESSION 2

**THURSDAY 20 OCTOBER 2016, 18.30-20.00**

2nd floor, Fra Angelico & Leonardo Rooms. For location of posters see opposite page.

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EXHIBITION HALL
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 m² leading regenerative medicine for everyone

www.euroclonegroup.it

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 iPSC cell bank with core and mirror facilities,
• the set-up of a robust and reliable supply chain for iPSC 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/ALIA).
Find out more about this large European public-private partnership at

www.ebisc.eu
Booth 12: Novasep is a contract manufacturing organisation specialised in the production of viruses & 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 & 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

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 10 vials) batches easily installable in BioSafety Cabinet or Isolator.
Application of this technology in Regenerative Medicine enables better cells viability and recovery, reduction of residual volume, 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, Stemmedica, 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 merckmillipore.com and sigma-aldrich.com.
www.merckmillipore.com • www.sigma-aldrich.com
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 iPSC. 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

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
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

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

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 iPSCs 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

Booth 30: BioReliance / SAFC: see page 30

www.bioreliance.com

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 pre-clinical 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
**Booth 34: ALS CellCelector™**

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 characterization (qPCR, sequencing, ...). CellCelector combines bright field, phase contrast 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**

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)**

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

**Booth 38: PSNResearch**

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**

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: RuRo**

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
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: IntelliCyte 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 ViroCyte 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

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 metastasis, 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 Île 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 (open to European students), laboratory small and large equipment and scientific workshops in the Paris Île de France region.
*DIM are « Domaines d’Intérêt Majeur », Major Interest Domains that were identified (2012-2017) by the French Region “Île de France” including Paris and its surroundings, to foster and 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.
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.
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, 8pm–1am
Live band 8–11pm, 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 Euros per person
2 drink vouchers per person to be collected from the ESGCT booth, see page 61
# PROGRAMME

## TUESDAY 18 OCTOBER 2016

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<td>09.00-09.20</td>
<td><strong>Planning a clinical trial</strong></td>
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<tr>
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<td>INV001 Considerations for clinical trials with cellular therapies</td>
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<td></td>
<td>Kim Champion, University College London</td>
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<tr>
<td>09.20-10.00</td>
<td><strong>Manufacturing of gene and cell products</strong></td>
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<td>INV002 What should be anticipated and implemented to ensure a successful process transfer. A CMO perspective with risk analysis from analytical to process transfer</td>
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<td>Francis Dupont, Novasep, Gosselies</td>
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<td>INV003 Development and manufacture of iPSC-derived cells to a specification: The CDI experience as a contractor</td>
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<td>Derek Hei, Cellular Dynamics International, Madison, WI</td>
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<td>10.00-10.20</td>
<td><strong>Gene and cell therapy technologies</strong></td>
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<td>10.10-10.20</td>
<td>INV004 Gene and cell therapy technologies</td>
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<td>Bobby Gaspar, University College London</td>
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<td>10.20-11.20</td>
<td><strong>Pricing and reimbursement</strong></td>
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<td>INV005 Early insights from NICE: ATMPs – evidence generation, evaluation, managed access</td>
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<td>Leezza Osipenko, NICE, London</td>
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<td>INV006 Hurdles in developing new Advanced Therapies: the experience with Holoclar</td>
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<td>Andrea Chiesi, Chiesi, Parma</td>
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<td>11.00-11.20</td>
<td>INV007 The Strimvelis experience</td>
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<td>Claude Schmitt, GSK, Brentford</td>
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<td>11.20-11.40</td>
<td><strong>Academic vs commercial clinical development strategy</strong></td>
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<td>11.20-11.40</td>
<td>INV008 Running clinical trials through a venture capital financed biotech company</td>
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<td>11.40-12.00</td>
<td>Regulatory strategy in gene and cell therapy development</td>
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<td>Chair: Guido Pantè, AIFA</td>
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## CLINICAL TRIAL AND COMMERCIALISATION WORKSHOP

Fourth Floor (Uccello Room)

- **Planning a clinical trial**
  - INV001 Considerations for clinical trials with cellular therapies
  - Kim Champion, University College London

- **Manufacturing of gene and cell products**
  - 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
  - INV003 Development and manufacture of iPSC-derived cells to a specification: The CDI experience as a contractor
  - Derek Hei, Cellular Dynamics International, Madison, WI

- **Gene and cell therapy technologies**
  - INV004 Gene and cell therapy technologies
  - Bobby Gaspar, University College London

- **Pricing and reimbursement**
  - INV005 Early insights from NICE: ATMPs – evidence generation, evaluation, managed access
  - Leezza Osipenko, NICE, London
  - INV006 Hurdles in developing new Advanced Therapies: the experience with Holoclar
  - Andrea Chiesi, Chiesi, Parma
  - INV007 The Strimvelis experience
  - Claude Schmitt, GSK, Brentford

- **Academic vs commercial clinical development strategy**
  - INV008 Running clinical trials through a venture capital financed biotech company
  - Kerry Fisher, University of Oxford

- **Regulatory strategy in gene and cell therapy development**
  - Chair: Guido Pantè, AIFA
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

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

10.30-10.00 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 vivo 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 – Passi Perduti

TUESDAY 18 OCTOBER 2016

EDUCATION DAY
Brunelleschi Auditorium

13.30-14.30 E4a: Immunotherapy & 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

### PUBLIC ENGAGEMENT DAY FOR PATIENT ASSOCIATIONS AND SCIENCE DIALOGUES WITH CITIZENS:
Leading edge therapies for rare diseases
First floor (Piero Della Francesca Room)

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<td>09.45-10.15</td>
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<td>09.45-10.15</td>
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<td>How to foster access to therapies</td>
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<td>10.15-10.45</td>
<td>Newborn screenings for metabolic diseases</td>
<td>Giancarlo La Marca, University of Florence</td>
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<td>Safety studies</td>
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<td>Use of animal models in research: why it is still a need</td>
<td>Giuliano Gignaschi, Istituto Di Ricerche Farmacologiche Mario Negri, Milan</td>
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<td>Science and bio-ethics</td>
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<td>Stem cells and new generation sequencing: new frontiers in medicine</td>
<td>Giuseppe Testa, IFOM-IEO Campus, Milan</td>
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| 11.45-13.00 | Panelists:                        | Nicola Spinelli Casacchia, President, Uniorno FIMR
     |                                   | Andrea Buzzu, President, Fondazione Paracelsa
     |                                   | Michele Lipucci, Eurordis
| 11.45-13.00 | Chairs:                          | Ilaria Bartoli Ciancaleoni, Osservatorio Malattie Rare
     |                                   | Alessia Daturi, Fondazione Telethon
| 13.00-14.00 | Lunch & networking (in the room) |                                 |
| 14.00-14.30 | New frontiers in science         |                                   |
| 14.30-16.30 | Role playing: science dialogues  | Anna Maria Zaccheddu, Fondazione Telethon |
| 16.30-17.00 | Closing remarks                  |                                 |

### MOLMED SYMPOSIUM
An entrepreneurial approach to translate academic knowledge into therapeutic solutions for all patients
Ground Floor (Botticelli Room)

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<tr>
<th>Time</th>
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<tr>
<td>12.30-14.00</td>
<td>Lunch and registration (in the room)</td>
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<tr>
<td>14.00-14.15</td>
<td>When pioneers in cell &amp; gene therapy come up with a ‘business’ idea</td>
<td>Claudia Bordignon, Molmed, Milan</td>
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<tr>
<td>14.15-14.30</td>
<td>Academia ready to be a productive partner for biotech companies</td>
<td>Fabio Ciceri, San Raffaele Scientific Institute, Milan</td>
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<tr>
<td>14.30-14.45</td>
<td>How the financial market operated and operates in sustaining the biotech sector development</td>
<td>Gil Bar-Nahum, Jefferies Global Healthcare Group, Bresso</td>
</tr>
<tr>
<td>14.45-15.00</td>
<td>A picture of the European biotech sector: strengths and weaknesses</td>
<td>Carlo Incerti, Europabi, Bresso</td>
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</table>
| 15.00-15.30 | Round Table                      | Andrea Cabrini – Class CNBC
     |                                   | Martin Andrews, GSK, Brentford
     |                                   | Gil Bar-Nahum, Global Healthcare Group at Jefferies International Limited
     |                                   | Claudia Bordignon, MolMed
     |                                   | Fabio Ciceri, San Raffaele Scientific Institute
     |                                   | Carlo Incerti, Europabi
     |                                   | Francesca Pasinelli, Telethon Foundation
| 16.30-17.00 | Closing remarks                  |                                 |
We are leading the gene therapy revolution with integrated product platforms encompassing gene therapy, cancer immunotherapy and gene editing - providing us with the potential to deliver one-time transformative therapies to patients with serious diseases.

Please visit us at www.bluebirdbio.com to learn more.

PROGRAMME
TUESDAY 18 OCTOBER 2016

MAIN CONGRESS

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

Molecular therapy ‘meet the editor’ reception
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 Schroeder, 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
Yen 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

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 (G5010) 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

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
Odd numbered posters available for viewing in Leonardo & Fra Angelico rooms

12.45-13.45
Michelangelo
Lunch Symposium: Regulatory workshop for ATMPs
BioReliance

13.45-13.55
Regulatory guidance and advice on the quality control of Advanced Therapy Medicinal Products
Martin Wisher, BioReliance

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
### PROGRAMME

#### WEDNESDAY 19 OCTOBER 2016

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<tr>
<td>14.00-14.30</td>
<td>INV038 Challenges of immaturity and proliferation in using hPSC-derived cardiomyocytes as disease models&lt;br&gt;Christine Mummery, Leiden University Medical Center, University of Twente</td>
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<tr>
<td>14.30-15.00</td>
<td>INV039 Small RNA therapy for cardiac regeneration&lt;br&gt;Mauro Giacca, International Centre for Genetic Engineering and Biotechnology (ICGEB), Trieste</td>
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<tr>
<td>15.00-15.30</td>
<td>INV040 Remuscularisation of injured hearts with human embryonic stem cell-derived cardiomyocytes&lt;br&gt;Michael Laflamme, University Health Network, Toronto</td>
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<td>15.30-16.00</td>
<td>INV041 Tricyclo-DNA: a new generation of antisense oligonucleotides for splice switching&lt;br&gt;Luis Garcia, Inserm UMR 1179, Paris</td>
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<tr>
<td>16.00-16.30</td>
<td>Coffee break</td>
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<tr>
<td>16.30-18.30</td>
<td>Parallel sessions 3a, 3b, 3c, 3d</td>
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<tr>
<td>16.30-18.30</td>
<td>Botticelli 3a: Organoids and high throughput platforms&lt;br&gt;Chairs: Valentina Greco, Melissa Little</td>
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<td>16.30-17.00</td>
<td>INV042 Generating 3D models of the human cerebral cortex to study development and disease&lt;br&gt;Sergiu Pasca, Stanford University, CA</td>
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<td>17.00-17.30</td>
<td>INV043 Detecting and killing pancreatic cancer&lt;br&gt;David Tuveson, Cold Spring Harbor Laboratory</td>
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<td>17.30-17.45</td>
<td>Proffered papers</td>
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<td>17.30-17.45</td>
<td>OR007 Generation of implantable 3D skeletal muscle tissue from human embryonic stem cells and muscular dystrophy IPS cells&lt;br&gt;Francesco Saverio Tedesco, University College London</td>
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<td>17.45-18.00</td>
<td>OR008 The development and characterization of rAAV vectors in patient-derived intestinal organoids and CF mice as a treatment for cystic fibrosis&lt;br&gt;Marianne Carlon, Laboratory for Molecular Virology and Drug Discovery, Division of Molecular Medicine, KU Leuven</td>
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<td>18.00-18.15</td>
<td>OR009 Isolating and characterising human cone photoreceptors for cell therapy&lt;br&gt;Emily Welby, University College London</td>
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<td>18.15-18.30</td>
<td>OR010 Assay development and high throughput screening in iPSCs-derived cortical glutamatergic neurons from two neurodevelopmental disorders caused by symmetrical dosage imbalance&lt;br&gt;Francesca Cavalla, University of Milan</td>
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<td>16.30-18.30</td>
<td>Masaccio 3b: Stem cell based neural disease modelling&lt;br&gt;Chairs: Angela Gritti, Paul Tesar</td>
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<td>16.30-17.00</td>
<td>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&lt;br&gt;Giuseppe Testa, University of Milan</td>
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<td>17.00-17.30</td>
<td>INV045 iPSC-based modelling of Parkinson’s disease&lt;br&gt;Angel Raya, Center for Regenerative Medicine, Barcelona</td>
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<td>17.30-17.45</td>
<td>Proffered papers</td>
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<td>17.30-17.45</td>
<td>OR011 Two factor fibroblast reprogramming generates induced Schwann cells with myelinogenic and nerve regenerating potential&lt;br&gt;Pietro Mazzara, SR-Tiger, Milan</td>
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<td>17.45-18.00</td>
<td>OR012 Neural stem cell transplantation in Parkinsonian mice triggers an astrocyte-dependent dopaminergic neurorestoration&lt;br&gt;Bianca Marchetti, University of Catania</td>
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<td>18.00-18.15</td>
<td>OR013 Leveraging pluripotent stem cells as a scalable platform to discover chemical therapeutics for genetic disorders of myelin&lt;br&gt;Matthew Ellis, Case Western Reserve University, Cleveland, OH</td>
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<tr>
<td>18.15-18.30</td>
<td>OR014 Mitochondrial disease phenotype in Friedreich’s ataxia patient iPSC-derived sensory neurons&lt;br&gt;Roxana Natt, Medical University Innsbruck</td>
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<td>16.30-18.30</td>
<td>Michelangelo 3c: Cardiovascular gene and cell therapy&lt;br&gt;Chairs: Seppo Ylä-Herttuala, Mauro Giacca</td>
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<td>16.30-17.00</td>
<td>INV046 Therapeutic vascular growth for cardiovascular diseases&lt;br&gt;Seppo Ylä-Herttuala, Al Virtanen Institute, Kuopio</td>
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<td>17.00-17.30</td>
<td>INV047 Non-coding RNA in vascular repair and regeneration&lt;br&gt;Andrew Baker, The University of Edinburgh</td>
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<td>Proffered papers</td>
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<td>17.30-17.45</td>
<td>OR015 Fixing the so-called unfixable: regenerating untreatable fixed myocardial scar in heart failure patients&lt;br&gt;Ajan Reginald, Cardiolife, Cardiff</td>
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<td>17.45-18.00</td>
<td>OR016 Using omics tools to improve differentiation and maturation of cardiomyocytes derived from human pluripotent stem cells&lt;br&gt;Paula Alves, iBET/ITQB, Oeiras</td>
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### WEDNESDAY 19 OCTOBER 2016

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<td>18.15-18.30</td>
<td>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</td>
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</table>
| 16.30-18.30   | 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 |
| 18.00-18.15   | Proffered papers  
  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 |
| 18.15-18.30   | OR020 Targeting the TCR β-constant region for specific immunotherapy of T-cell malignancies  
  Paul Maciocia, University College London |
| 18.15-18.30   | OR021 Immunovirotherapy in combination with immune checkpoint inhibitors for treating glioblastoma stem cell-derived tumors  
  Samuel Robkin, 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 |
| 20.00-23.00   | Speaker dinner (by invitation only)  
  Walking party departs at 20.00 from congress registration area (main entrance) |

Committed to developing effective gene therapies that transform the lives of patients

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.
### THURSDAY 20 OCTOBER 2016

#### MAIN CONGRESS

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<td>08.00-10.00</td>
<td>4: Cancer immuno–gene therapy</td>
<td><strong>Chairs:</strong> Katherine High, Attilio Bondanza</td>
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<td>08.00-08.30</td>
<td>INV050 CAR T-cell therapy: from CD19 to other targets</td>
<td>Gianpietro Dotti, Department of Microbiology and Immunology, UNC Chapel Hill</td>
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<td>08.30-09.00</td>
<td>INV051 Chimeric antigen receptor T-cells - killing cancer by design</td>
<td>Stanley Riddell, Fred Hutchinson Cancer Research Center, University of Washington, Seattle</td>
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<td>09.00-09.30</td>
<td>INV052 Engineering T-cells for cancer therapy</td>
<td>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</td>
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<tr>
<td>09.30-10.00</td>
<td>INV053 TCR gene edited memory stem T cells for cancer immunotherapy</td>
<td>Chiara Bonini, Università Vita-Salute San Raffaele and Ospedale San Raffaele Scientific Institute, Milan</td>
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<tr>
<td>10.00-10.30</td>
<td>Coffee break</td>
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<tr>
<td>10.30-12.30</td>
<td>Botticelli: MSC gene and cell therapy</td>
<td><strong>Chairs:</strong> Michael Laflamme, Luis Garcia</td>
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<td>10.30-11.00</td>
<td>INV056 Use of expanded adipose stem cells in the treatment of inflammatory diseases</td>
<td>Wilfried Dalemans, Tigenix NV, Hasselt</td>
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<tr>
<td>11.00-11.30</td>
<td>INV057 Therapeutic immune regulation by mesenchymal stromal cells</td>
<td>Willem Fibbe, Leiden University Medical Center</td>
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<td>11.30-11.45</td>
<td>Proffered papers</td>
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<tr>
<td>11.45-12.00</td>
<td>OR027 Comprehensive characterisation of bone marrow-derived mesenchymal stromal cells from patients affected by primary immunodeficiency</td>
<td>Nadia Starc, Bambino Gesù Children's Hospital, Rome</td>
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<td>12.00-12.15</td>
<td>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</td>
<td>Sabine Geiger, Apceth GmbH &amp; Co. KG, Munich</td>
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<td>12.15-12.30</td>
<td>OR029 Mesenchymal stromal cells prevent graft failure in a mouse model of hematopoietic stem cell gene therapy</td>
<td>Maria Fernandez Garcia, CIEMAT/CIBERER, IIS-F JD, UAM, Madrid</td>
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<td>12.30-12.45</td>
<td>OR030 Pulmonary artery reconstruction using cord blood-derived multipotent stem cells in vitro and in vivo study</td>
<td>Huudong Jia, University of Bristol</td>
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<td>10.30-12.30</td>
<td>Masaccio: In vivo gene therapy</td>
<td><strong>Chairs:</strong> Xavier Anguela, Hildegard Büening</td>
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<td>10.30-11.00</td>
<td>OR058 Assessing and modulating immunogenicity in AAV vector mediated gene transfer</td>
<td>Federico Mingozzi, Genethon, Evry</td>
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<tr>
<td>11.00-11.30</td>
<td>OR059 Gene therapy for haemophilia</td>
<td>Katherine High, Spark Therapeutics, Philadelphia, PA</td>
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## PROGRAMME

**THURSDAY 20 OCTOBER 2016**

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<tr>
<td>11.30-11.45</td>
<td>OR031</td>
<td><strong>Proffered papers</strong>&lt;br&gt;Sustained expression with partial correction of neutrophil defects 5 years after intramuscular raaV1 gene therapy for alpha-1 antitrypsin deficiency</td>
<td>Terry Flotte, University of Massachusetts Medical School, Worcester</td>
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<td>11.45-12.00</td>
<td>OR032</td>
<td><strong>Liver-directed gene therapy with lentiviral vectors in animal models of haemophilia B</strong></td>
<td>Michela Milani, SR-Tiget, Vita-Salute San Raffaele University Milan</td>
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<tr>
<td>12.00-12.15</td>
<td>OR033</td>
<td><strong>Towards clinical gene and cell therapies for OPMD</strong></td>
<td>Capucine Trollet, UPMC Univ Paris 06, UM76, INSERM U974, Institut de Myologie, CNRS FRE3617</td>
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<tr>
<td>12.15-12.30</td>
<td>OR034</td>
<td><strong>Red blood cells as therapeutic carrier in monogenic disorders</strong></td>
<td>Giuseppa Piras, University College London</td>
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<td>12.30-14.00</td>
<td></td>
<td><strong>Lunch</strong>&lt;br&gt;Even numbered posters available for viewing in Leonardo &amp; Fra Angelico rooms</td>
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<tr>
<td>14.00-16.00</td>
<td><strong>Masaccio</strong>&lt;br&gt;5a</td>
<td><strong>Proffered papers</strong>&lt;br&gt;<em>Cancer stem cells</em>&lt;br&gt;Chairs: John Dick, Emmanuelle Passegue</td>
<td>Michael Clarke, Stanford University, CA</td>
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<tr>
<td>14.00-14.30</td>
<td>INV060</td>
<td><strong>Stem cells in cancer and regenerative medicine</strong>&lt;br&gt;</td>
<td>John Dick, Emmanuelle Passegue, Stanford University, CA</td>
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<td>14.30-15.00</td>
<td>INV061</td>
<td><strong>Leukemic stem cell interactions with the microenvironment: friend or foe?</strong>&lt;br&gt;Chairs: John Dick, Emmanuelle Passegue</td>
<td>Dominique Bonnet, The Francis Crick Institute, London</td>
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<tr>
<td>14.30-15.00</td>
<td>INV062</td>
<td><strong>Plasticity of cancer cells: lessons from glioblastomas</strong></td>
<td>Inder Verma, The Salk Institute, La Jolla, CA</td>
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<td>15.00-15.15</td>
<td>OR035</td>
<td><strong>microRNA-126 orchestrates a stem cell-like programme in Acute B Lymphoblastic Leukemia (B-ALL)</strong>&lt;br&gt;Chairs: John Dick, Emmanuelle Passegue</td>
<td>Carolina Caserta, SR-Tiget, Vita-Salute San Raffaele University Milan</td>
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<tr>
<td>15.15-15.30</td>
<td>OR036</td>
<td><strong>A stem cell oriented phylogeny of cancers derived novel cancer gene expression signature in all undifferentiated cancers as a therapeutic target</strong>&lt;br&gt;Chairs: John Dick, Emmanuelle Passegue</td>
<td>Robert Downey, Memorial Sloan Kettering Cancer Center, New York City</td>
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<tr>
<td>14.00-16.00</td>
<td><strong>Auditorium</strong>&lt;br&gt;5b</td>
<td><strong>Ex vivo HSC based gene and cell therapy</strong>&lt;br&gt;Chairs: Juan Bueroen, Guisanna Ferrari</td>
<td>Don Kohn, University of California, Los Angeles, CA</td>
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<tr>
<td>14.00-14.30</td>
<td>INV063</td>
<td><strong>Gene therapy for primary immune deficiencies: ADA-SCID and XCGD</strong>&lt;br&gt;Chairs: Juan Bueroen, Guisanna Ferrari</td>
<td>Don Kohn, University of California, Los Angeles, CA</td>
</tr>
</tbody>
</table>

**Parallel sessions 5a, 5b, 5c**

- **5a: Cancer stem cells**
  - Chairs: John Dick, Emmanuelle Passegue
- **5b: Ex vivo HSC based gene and cell therapy**
  - Chairs: Juan Bueroen, Guisanna Ferrari
- **5c: DNA based gene transfer and in vivo**
  - Chairs: Zoltan Ivics, Amber Salzman
THURSDAY 20 OCTOBER 2016

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tr>
<td>16.00-16.30</td>
<td>Coffee break</td>
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<tr>
<td>16.30-18.30</td>
<td><em>S: New technologies: targeted genome and epigenome editing, new vector design, organoids</em>&lt;br&gt;Chairs: Christine Mummery, George Q Daley</td>
</tr>
<tr>
<td>16.30-17.00</td>
<td><em>INV066 Inheritable silencing of endogenous genes by hit-and-run targeted epigenetic editing</em>&lt;br&gt;Angelo Lombardo, SR-Tiget, Milan</td>
</tr>
<tr>
<td>17.00-17.30</td>
<td><em>INV067 Genome engineering: prospects and challenges</em>&lt;br&gt;Feng Zhang, MIT, Cambridge, MA</td>
</tr>
<tr>
<td>17.30-18.00</td>
<td><em>INV068 Generating a kidney from human pluripotent stem cells: where to from here?</em>&lt;br&gt;Melissa Little, Murdoch Children’s Research Institute, University of Melbourne</td>
</tr>
<tr>
<td>18.00-18.30</td>
<td><em>INV069 From pluripotent stem cells to cortical circuits</em>&lt;br&gt;Pierre Vanderhaeghen, Université Libre de Bruxelles</td>
</tr>
<tr>
<td>18.30-20.00</td>
<td>Poster session 2 (Even poster numbers). See page 44 for details</td>
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<tr>
<td>20.30-01.00</td>
<td>Molecular Mingle evening – Mercato Centrale. See page 66</td>
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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

1. Product research and development
   - Regulatory and clinical expertise
   - Four product candidates approved for the clinic
   - One further product-approved for the clinic before end of 2016

2. IP Ownership
   - Corporate know how
   - Trade secrets, materials
   - Proprietary analytics
   - Lentiviral vector patent estate

3. Expertise
   - 20 years experience in lentiviral vector development
   - Expert staff
   - Three independent GMP production suites
   - Process development and scale-up
   - Laboratories for GMP, GLP and GCLP analytical testing
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<tr>
<th>Time</th>
<th>Session</th>
<th>Topic</th>
<th>Chairs</th>
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<tbody>
<tr>
<td>09.00-10.30</td>
<td>6a</td>
<td>RNA based gene transfer and integration studies</td>
<td>Chairs: Eugenio Montini, Terry Flotte</td>
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<tr>
<td>09.00-09.30</td>
<td></td>
<td>INV071 Clonal tracking of engineered human hematopoiesis through</td>
<td>Luca Biasco, SR-Tiget, Milan, Gene Therapy</td>
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<td></td>
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<td>integration sites analysis</td>
<td>Programme Dana-Farber/Boston Children's</td>
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<td>INV070 Wherever you go – there you are: tracking DNA modifications</td>
<td>Clonal tracking of engineered human</td>
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<td>hematopoiesis through integration sites</td>
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<td>INV072 Highly efficient gene editing in hematopoietic stem cells</td>
<td>Toni Cathomen, University Medical Centre,</td>
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<td>INV073 Genome editing for Duchenne muscular dystrophy</td>
<td>Freiburg</td>
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<td>OR047 New molecular surrogate assay for genotoxicity assessment</td>
<td>Michael Rathe, Hannover Medical School</td>
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<td>OR048 Identification and ranking of different chromatin insulators to</td>
<td>block vector-driven enhancer-mediated</td>
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<td></td>
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<td>OR051 Clinical update, molecular analyses, and proposed</td>
<td>insertion mutagenesis in vivo</td>
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<td>OR052 Anti-tumor potency of cancer vaccine ONCOS-102 in the treatment</td>
<td>Clinical update, molecular analyses, and</td>
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<td>OR053 Intrinsic defect in Was-/- platelets: studies in conditional</td>
<td>proposed mechanism of action of Toca 511</td>
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<td>OR054 Gene therapy for Ebola virus infections based on AAV vectors</td>
<td>A retroviral replicating vector in three</td>
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<tr>
<td>09.00-10.30</td>
<td>6b</td>
<td>Genome editing and gene correction</td>
<td>ascending dose trials in patients with</td>
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<tr>
<td>09.00-09.30</td>
<td></td>
<td>INV074 Cancer virotherapy with oncolytic adenoviruses</td>
<td>recurrent high-grade glioma</td>
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<tr>
<td>09.30-10.00</td>
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<td>INV075 Development of prostate cancer gene therapy in Japan</td>
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<tr>
<td>09.00-10.30</td>
<td>6c</td>
<td>Cancer gene therapy</td>
<td>Chairs: Len Seymour, Inder Verma</td>
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<tr>
<td>09.00-09.30</td>
<td></td>
<td>INV076 Gene therapy for hereditary and acquired life-threatening,</td>
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<td>09.30-10.00</td>
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<td>INV077 Gene therapy-based approach for immune tolerance induction</td>
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<td>10.00-10.15</td>
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<td>OR049 Targeted gene therapy in the treatment of X-Linked Hyper-IgM</td>
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<td>10.15-10.30</td>
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<td>OR050 Identification of high-fidelity Cas9 variants using a yeast-based</td>
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<tr>
<td>11.00-12.00</td>
<td>6d</td>
<td>Gene therapy in the market</td>
<td>Chairs: Luigi Naldini, Sven Kili</td>
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<tr>
<td>09.00-09.30</td>
<td></td>
<td>INV078 Primary immune deficiencies: a natural target for ex vivo</td>
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<tr>
<td>09.30-10.00</td>
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<td>INV079 Ex vivo gene therapy in ADA-SCID: clinical data and experiences</td>
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<tr>
<td>11.00-11.15</td>
<td></td>
<td>INV080 Translating experimental gene therapy into clinical reality</td>
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<td>11.15-11.30</td>
<td></td>
<td>INV081 Patient management through the gene therapy process</td>
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<tr>
<td>11.30-11.45</td>
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<tr>
<td>11.45-12.00</td>
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## Friday, 21 October 2016

### 12:00-13:00

<table>
<thead>
<tr>
<th>Time</th>
<th>Location</th>
<th>Session</th>
<th>Chair(s)</th>
<th>Presenters</th>
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</thead>
<tbody>
<tr>
<td>12.00-12.30</td>
<td>Auditorium</td>
<td>Inv082: Gene therapy of mucopolysaccharidosis VI</td>
<td>Alberto Auricchio, Tigern, Naples</td>
<td></td>
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<tr>
<td>12.30-13.00</td>
<td>Auditorium</td>
<td>Inv083: Making a good vector even better: novel rAAVs for classical gene therapy and genome editing</td>
<td>Mark Kay, Stanford University, CA</td>
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<tr>
<td>13.00-14.00</td>
<td>Limonaria, Passi Perduti</td>
<td>Lunch</td>
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### Parallel Sessions 7a, 7b, 7c

<table>
<thead>
<tr>
<th>Time</th>
<th>Location</th>
<th>Session</th>
<th>Chair(s)</th>
<th>Presenters</th>
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</thead>
<tbody>
<tr>
<td>14.00-14.30</td>
<td>Auditorium</td>
<td>Inv084: T-Cell and cancer immunotherapy</td>
<td>Dirk Busch, Technische Universität München</td>
<td></td>
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<tr>
<td>14.30-15.00</td>
<td></td>
<td>Inv085: Macrophage-based delivery of immunostimulatory and antiangiogenic molecules into the tumor microenvironment</td>
<td>Bernhard Gentner, SR-Tiget, Milan</td>
<td></td>
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<tr>
<td>15.00-15.15</td>
<td></td>
<td>Or055: Reduced CAR tonic signaling and methods to enhance memory T cells result in improved in vivo efficacy in human multiple myeloma xenograft models</td>
<td>Richard Morgan, bluebirdbio</td>
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<tr>
<td>15.15-15.30</td>
<td></td>
<td>Or056: Balance of Anti-CD123 Chimeric Antigen Receptor (CAR) binding affinity and density in an in vitro model of acute myeloid leukemia</td>
<td>Sarah Tetramani, Universita’ Milano Bicocca, Osp. San Gerardo/Fondazione MBBM, Monza</td>
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<tr>
<td>15.00-15.30</td>
<td>Michelangelo</td>
<td>Inv086: ZFN-mediated genome editing in the liver – towards the correction of lysosomal storage diseases</td>
<td>Michael Holmes, Sangamo BioSciences Inc, Richmond, CA</td>
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<tr>
<td>15.15-15.30</td>
<td>Michelangelo</td>
<td>Inv087: MicroRNA-based therapeutics in cancer</td>
<td>Frank Slack, Harvard Medical School, Cambridge, MA</td>
<td></td>
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<tr>
<td>14.00-15.30</td>
<td>Michelangelo</td>
<td>Inv089: Mastering the challenges of manufacturing: the critical roles of closed systems and automation</td>
<td>Ian Johnston, Miltentyi Biotec, Bergisch Gladbach</td>
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<tr>
<td>15.00-15.30</td>
<td>Masaccio</td>
<td>Or057: Transcriptional silencing via synthetic DNA binding protein lacking canonical repressor domains as a potent tool to generate therapeutics</td>
<td>Salvatore Batta, Tigern, Naples</td>
<td></td>
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<tr>
<td>15.00-15.30</td>
<td>Masaccio</td>
<td>Or058: ASO-mediated Dnm2 knockdown prevents and reverts Myotubular myopathy in vivo in mice</td>
<td>Belinda Cowling, IGBMC, Strasbourg</td>
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<tr>
<td>14.00-15.30</td>
<td>Masaccio</td>
<td>Or059: Improving the purity of Adeno-associated viral vector preparations using DNA minicircle technology</td>
<td>Hildegard Bünning, University of Cologne, DZIF, University Hospital Cologne, Hannover Medical School</td>
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<tr>
<td>14.00-15.30</td>
<td>Masaccio</td>
<td>Or060: Staurosporine Increases Lentiviral Transduction of Human CD34+ Cells</td>
<td>Melissa Bonner, bluebirdbio, Cambridge, MA</td>
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<tr>
<td>15.15-15.30</td>
<td>Masaccio</td>
<td>Inv090: AAV-CYP46A1 brain administration restores cholesterol metabolism and is neuroprotective in Huntington’s disease</td>
<td>Nathalie Carrier-Lacave, INSERM UMR1169, Université Paris-Sud, CEA, DSV, FBM, MIRCen, Fontenay-aux-Roses</td>
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<tr>
<td>15.15-15.30</td>
<td>Masaccio</td>
<td>Inv091: AVXS-101 phase 1 gene therapy clinical trial in SMA type 1</td>
<td>Jerry Mendell, Nationwide Children’s Hospital, Ohio State University</td>
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<tr>
<td>15.15-15.30</td>
<td>Masaccio</td>
<td>Or061: From bench to bedside: A novel approach in the treatment of SMA Type 1 with gene therapy</td>
<td>Brian Kaspar, Nationwide Children’s Hospital, The Ohio State University Medical Center, AveXis, Inc</td>
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<tr>
<td>15.15-15.30</td>
<td>Masaccio</td>
<td>Or062: Improvements in motor function and PET findings following gene transfer to the patients with AADC deficiency</td>
<td>Taka Yamagata, Jichi Medical University</td>
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# PROGRAMME

**FRIDAY 21 OCTOBER 2016**

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<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>15.30-15.50</td>
<td>Coffee break</td>
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<tr>
<td>15.50-17.45</td>
<td>Presidential symposium and awards ceremony</td>
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<tr>
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<td><strong>Chairs</strong>: Nathalie Cartier-Lacave, Luigi Naldini</td>
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<tr>
<td>15.50-16.15</td>
<td>ESGCT AGM</td>
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</table>
| 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 | Germline editing debate                                   |
|           | **Chairs**: Roberto Buccione                               |
|           | Annelien Bredenoord, University Medical Center Utrecht    |
|           | Giuseppe Testa, 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, Mass     |
| 19.00-20.00 | Closing drinks                                             |

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**BAYER HEMOPHILIA AWARDS PROGRAM**

**SUPPORTING HEMOPHILIA RESEARCH, TREATMENT AND EDUCATION AROUND THE WORLD**

The Bayer Hemophilia Awards Program is our flagship hemophilia and hemostasis grants program, underlining our commitment to driving forward the scientific understanding of hemophilia and hemostasis, and to improving care for patients around the world.

Since 2003, over 270 grants have been awarded to clinicians, researchers and caregivers from 32 countries.

For more information, visit:  
[www.bayer-hemophilia-awards.com](http://www.bayer-hemophilia-awards.com)
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 cosmopolitan 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 variety 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 minimum 10 participants; tours around Florence require minimum 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 butcher 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 Baptistery.
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. 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 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 Team

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.

On the cutting edge of CNS gene therapy science Lysogene is currently expanding its pipeline to other genetic diseases affecting the central nervous system.

Lysogene is proud to support the ESGCT and its members
Spark Therapeutics is developing potential one-time gene therapies that re-imagine the treatment of debilitating diseases and transform the lives of patients.

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

MAKE A DONATION

TELETHON.FR

Please visit www.sparktx.com to learn more
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
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 some information and contact details for national societies below 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**

http://dg-gt.de
hildegard.buening@uk-koeln.de
Next meeting:
ESGCT Collaborative Congress with the DG-GT
Berlin, Germany
17–20 October 2017

**Finland**

http://fsgt.fi

**France**

www.sftcg.fr
office@sftcg.fr
Next meeting:
ESGCT Collaborative Congress with the SFTCG
Lausanne, Switzerland 2018

**The Netherlands**

http://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
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.

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 Aseguiñolaza, 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 Rio, 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 & cell therapy

Contact office@esgct.eu for details