European Society of Gene and Cell Therapy
Spring School 2016

20-22 April 2016
Stephansstift, Hannover, Germany

www.esgct.eu
Welcome to....

Das Stephansstift

Stephansstift, Kirchröder Str. 44, Hannover

The Stephansstift is quietly located in Hannover's Kleefeld district, close to the Hannover Medical Park. It is 15 minutes from the city centre by tram. Immersed in nature, the site is on the edge of the large city forest Eilenriede, and is located near the Hermann-Löns-Park and the Tiergarten; Hannover's deer park.
Tel. +49 511 53530
www.stephansstift.de

Registration & Information Desk

For registration and information regarding the Spring School:
Wednesday 20 April 09.00–18.00
Thursday 21 April 08.00–18.30
Friday 22 April 08.30–13.00

Information boards

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

In case of emergency, contact:
Gaëlle Jamar, Event Manager
Tel: +44 7766 475379
Email: office@esgct.eu

Taxis

Arnemann GmbH: +49 511 4584545
Gruß Taxen GmbH: +49 511 664964
Hallo Taxi 3811: +49 511 3811

Buses

Stop: Nackenberg
Bus nos.: 123, 124

Tram

Stop: Nackenberg (lines 4 and 5)

For more information about visiting Hannover see
www.hannover.de/en/Visit-Hannover

Organisers:
WATS.ON Ltd. www.wats-on.co.uk

Getting social!

Follow our official channels:

Facebook: www.facebook.com/esgct
Twitter: www.twitter.com/esgct
Instagram: www.instagram.com/esgct

Speaker hotel information

Mercure Hotel Hannover Medical Park
Feodor-Lynen-Str. 1, 30625 Hannover
Tel: (+49)511/95660
Email: H1631@accor.com
http://goo.gl/3g5Dj4

Book design based on Congress programmes produced by Catherine Charnock Creative:
www.catherinecharnock.co.uk
The Spring School is organised with the support of the following partners

**Principal Partners**

- European Society of Gene & Cell Therapy
- DG-GT e.V.
- DZIF
- MHH Hannover Medical School

**Partners and Supporters**

- EUFETS
- Human Gene Therapy
- PlasmidFactory
- PROGEN Biotechnik
- rebirth Cluster of Excellence
- SFB 738
Dear colleagues

On behalf of the European Society of Gene and Cell Therapy (ESGCT), the German Society for Gene Therapy (Deutsche Gesellschaft für Gentherapie e.V., DG-GT e.V.), the German Center for Infection Research (Deutsches Zentrum für Infekionsforschung) and Hannover Medical School (Medizinische Hochschule Hannover, MHH) it is my pleasure to welcome you to the beautiful city of Hannover for the first Spring School of the ESGCT.

We are witnessing an exciting time in Gene Therapy. What started as a fascinating idea was followed by years of tremendous efforts, both in basic and translational research, which lead to the first clear evidence of Gene Therapy as a cure for patients. However, these initial proofs confirming the concept of Gene Therapy as valid were overshadowed by the occurrence of severe side effects in some of the treated patients. Although we have to admit that we still have to decipher disease-related prerequisites for their occurrence, these side effects also fostered cross-border collaborations, enormous technological improvements and better understanding of the principles of tissue regeneration and of immunological responses towards Cell and Gene Therapy. These efforts resulted in clinical trials which clearly demonstrated the therapeutic efficacy of Gene and Cell Therapy and also more widespread applications, which today not only include concepts for the treatment of severe combined immune deficiency syndromes, but also of acquired and chronic diseases such as cancer, heart failure, neurodegenerative or metabolic diseases. While we still lack definite proof of long-term safety and therapeutic efficacy, it is evident that Gene Therapy has moved from a concept to clinical reality, with a clear impact on translational biomedicine. Also, further disciplines, such as Regenerative Medicine, or – as is the focus of this Spring School – Infection Research, have benefited from technologies and know-how initially developed for Cell and Gene Therapy.
With this Spring School we invite you to enter this exciting and fascinating area. You will gain insight on the current state of gene, cell and oncolytic therapy, learn secrets on how to tailor gene delivery tools and on novel strategies for treatments of inherited and acquired diseases, in vaccine development and in immunotherapy. Moreover, we encourage you to use this unique opportunity as inspiring platform for your own research and for networking.

Supporting you and your scientific career is part of our mission. Therefore, the ESGCT has teamed up with the above mentioned societies and institutions to launch this initiative, which will become a constant part of our educational efforts. This year’s meeting venue is the Stephansstift, nicely located at the edge of the Eilenriede. On behalf of all partners, I would like to thank the whole organizing team and all sponsors for their valuable contribution, without whom this event would not have been possible.

We very much look forward to your participation, and hope that you will enjoy three inspiring days at the first Spring School of the ESGCT.

Sincerely yours,

Prof. Dr. Hildegard Büning
ESGCT General Secretary
Spring School Organizing Committee
CHANGING THE FACE OF MODERN MEDICINE:
STEM CELLS & GENE THERAPY

FLORENCE
ITALY

18–21 OCTOBER 2016
The European Society of Gene & Cell Therapy warmly invites you to join us in Florence, Italy, for our annual meeting in 2016, in collaboration with the ISSCR and ABCD

KEYNOTE SPEAKER: HANS CLEVERS

6 PLENARY SESSIONS
Topics will range from biology to clinical application of cell and gene therapy in the following areas:
- Hematopoietic stem cells
- Skeletal and cardiac muscle stem cells
- Neural stem cells
- Cancer immuno-gene therapy
- New gene editing technologies and organoids
- In vivo liver-directed gene therapy

CONFIRMED PLENARY SPEAKERS:
John Dick, Len Zon, Luigi Naldini, Marina Cavazzana, Christine Mummery, Mauro Giacca, Michael Laflamme, Luis Garcia, Fred H. (Rusty) Gage, Masaio Takahashi, Malin Parmar, Paul Tésar, Ton Schumacher, Carl June, Stanley Riddell, Chiara Bonini, Feng Zhang, Angelo Lombardo, Melissa Little, Pierre Vanderhaeghen, Amit Nathwani, Alberto Auricchio, Mark A Kay
The European Society of Gene and Cell Therapy (ESGCT) promotes basic and clinical research in gene therapy, cell therapy, and genetic vaccines by facilitating education, the exchange of information and technology and by serving as a professional adviser to stakeholder communities and regulatory bodies in Europe.
www.esgct.eu

The German Society for Gene Therapy (DG-GT) is an association of scientists and clinicians who deal with issues of experimental and clinical gene therapy.
Our mission is to provide a network that promotes academic exchanges at all levels and participates in close cooperation with other national societies active in the translation of gene therapy approaches of experimental research to clinical applications.
The DG-GT is also an information platform and discussion forum for issues in the fields of gene therapy, molecular medicine, stem cell biology, and new molecular techniques.
www.dg-gt.de

The mission of the German Centre for Infection Research (DZIF) is to coordinate and strategically align translational infection research with the aim of developing new diagnostic, preventative and therapeutic methods for treating infectious diseases. In addition to its Thematic Translational Units (TTUs) and Technical Infrastructures (TIs), the DZIF comprises an Academy which aims to encourage, train and support the next generation of clinician scientists to strengthen translational infection research, e.g. by providing stipends to allow protected research time.
www.dzif.de

The Hannover Medical School (Medizinische Hochschule Hannover, MHH), founded in 1965, is one of the world’s leading university medical centres. Our research and patient care set national and international standards. We are also part of an excellent regional medical network. Due to its interdisciplinary research MHH has strong collaborative links with many academic and industrial research organizations worldwide. MHH concentrates its research activities to unravel basic mechanisms which will be, in close collaboration with clinical facilities, translated into clinical research. Building on a broad expertise in medicine, transplantation, immunology, genetics, biomedical implants and infectiology MHH scientists study human diseases and develop innovative strategies for diagnosis, prevention and therapy.
www.mh-hannover.de
EUFETS, a German-based company, supports the development and commercialisation of cell and gene therapies. Services include cGMP-compliant manufacturing of viral vectors, genetically modified cells and, as a new service, in vitro transcribed RNA. EUFETS offers process and assay development, validation, quality control, storage and QP release. EUFETS also supports preclinical product development (R&D/GLP studies) of biologics with customised in vitro bioanalytical programmes.

www.eufets.com

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

www.liebertpub.com/hum

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

www.plasmidfactory.com

PROGEN Biotechnik GmbH has been operating for years in the in vitro diagnostic fields like microbiology, infectious disease serology, immunology, as well as in biomedical and cell biology research with antibodies, reagents and tools for use in fields such as gene therapy research, antibody phage display technology, recombinant antibody engineering, and lipase activity. The company has a well-established reputation in the manufacture of antibodies, purified native and recombinant polypeptides and of in vitro diagnostic tests for niche markets. Progen is also a distributor of research reagents from several foreign companies in the German market.

http://www.progen.de/
The REBIRTH Cluster of Excellence (From Regenerative Biology to Reconstructive Therapy) is an internationally renowned institution for regenerative medicine. REBIRTH is funded since 2006 by the Excellence Initiative of the German Federal and State Governments. A network of over 30 institutes with 60 different work groups and over 250 researchers are developing gene- and cell-based therapeutic strategies for diseases of the heart, lungs, liver and blood through interdisciplinary collaboration between the various scientific disciplines integrated within REBIRTH. By drawing on the knowledge gained from fundamental research conducted within REBIRTH and its translation into experimental medicine, and by identifying relevant mechanisms involved in regenerative processes in the human body REBIRTH aims to develop new approaches and technologies for medical use. These will be applied in everyday clinical routine for the benefit of patients implemented for example at Hannover Medical School.

http://www.rebirth-hannover.de/

Although there has been significant progress over the last 100 years many cures for diseases remain to be found. Cancer and hepatitis are just 2 examples. Diseases can lead to dysfunctional organs. Although there are many options for treatment sometimes a transplantation is inevitable. A new functional organ can save a patient’s life. Despite all progress in transplantation medicine, it remains one of the most complex fields of medicine. Patients with a transplant are dependent on immunosuppressive medication which prevents the human body to reject the new organ. We are working on improving this form of medication so the side effects will decrease. Another goal is to ultimately increase the longevity of organs for a higher quality of life for our patients.

The Collaborative Research Center 738 researches the optimization of conventional and innovative transplants. What does this mean exactly? A transplant is an organ removed from one body and transplanted into another. Organ transplantation is mostly associated with solid organs such as liver, kidneys, heart and lung. A less known fact is that bone marrow can be transplanted as well to treat diseases like leukemia. The procedure of a transplant is complex and there are risks and side effects. A transplanted organ can be rejected by the recipient’s body because our immune system is trained to identify anything that is not part of our own body. One of our goals is to prevent this rejection by giving the patients immunosuppressive medication. The disadvantage of this medication is a lower defense against viruses. We are trying to improve these medications so they ultimately prevent the human body from rejecting the new organ while maintaining the ability to fight diseases and viruses.

www.sfb738.de
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<tr>
<th>Time</th>
<th>Session</th>
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<tr>
<td>9.30-12.00</td>
<td>An Introduction to Public Engagement for Researchers (optional workshop)</td>
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<td>What is public engagement, why is it important, and how you can do it</td>
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<td><em>WATS.ON Ltd.</em> (advance booking required by emailing <a href="mailto:abstracts@esgct.eu">abstracts@esgct.eu</a>)*</td>
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<tr>
<td>13.00-13.30</td>
<td>Welcome Address</td>
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<td>Hannover Medical School</td>
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<td><em>Christopher Baum, Presidium, Hannover Medical School</em></td>
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<td>European Society for Gene and Cell Therapy</td>
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<td></td>
<td><em>Nathalie Cartier, INSERM U986, Fontenay aux Roses</em></td>
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<td>Organizing Committee</td>
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<td><em>Hildegard Büning, Institute of Experimental Hematology, Hannover Medical School</em></td>
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<tr>
<td>13.30-15.25</td>
<td>Gene and Cell Therapy - past, present and beyond</td>
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<td>13.30-14.05</td>
<td>Gene Therapy - Current Status and Future Direction</td>
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<td><em>Luigi Naldini, San Raffaele Telethon Institute for Gene Therapy (TIGET), San Raffaele Scientific Institute</em></td>
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<tr>
<td>14.05-14.40</td>
<td>Stem Cell Therapy for Retinal Degeneration</td>
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<td><em>Robin Ali, Institute of Ophthalmology, University College London</em></td>
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<tr>
<td>14.40-15.25</td>
<td>Virotherapy - Current Status and Future Direction</td>
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<td><em>Len Seymour, Department Oncology, University of Oxford</em></td>
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<tr>
<td>15.25-15.45</td>
<td>Coffee Break</td>
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<td>15.45-17.20</td>
<td>News from the tool box (I)</td>
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<tr>
<td>15.45-16.20</td>
<td>Retroviral vectors: Fascinating tools for gene therapy and cell fate control</td>
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<td><em>Axel Schambach, Institute of Experimental Hematology, Hannover Medical School</em></td>
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<tr>
<td>16.20-16.55</td>
<td>Engineering AAV vectors to optimize host-vector-interaction</td>
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<td><em>Hildegard Büning, Institute of Experimental Hematology, Hannover Medical School</em></td>
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<tr>
<td>16.55-17.30</td>
<td>Vectorizing genetic engineering tools</td>
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<td><em>Dirk Grimm, Department of Infection Diseases, Heidelberg University</em></td>
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<tr>
<td>18.00-19.00</td>
<td>Dinner</td>
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<tr>
<td>19.30</td>
<td>After Dinner Speech</td>
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<td><em>Len Seymour, Department Oncology, University of Oxford</em></td>
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<td>Successful Conference Presentations and Networking in a &quot;Nutshell&quot;. A short interactive lecture/workshop on the essential principles of excellent scientific communication for your research and career success.</td>
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<td><em>Dr. Paul Charlton, DZIF guest faculty; <a href="http://www.dzif.de/en/academy/guest_faculty">www.dzif.de/en/academy/guest_faculty</a></em></td>
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<tr>
<td>8.30-10.15</td>
<td>News from the tool box (II)</td>
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</table>
| 8.30-9.05 | Recent progress in CNS gene therapy: applications to neurodegenerative diseases  
  *Nathalie Cartier, INSERM U986, Fontenay aux Roses*                      |
| 9.05-9.40 | Sleeping Beauty Transposon-Based Non-Viral Gene Delivery for Molecular Medicine  
  *Zoltan Ivics, Paul-Ehrlich-Institut, Langen*                            |
| 9.40-10.15 | Next generation muscle-directed gene therapy using skeletal-muscle specific transcriptional modules identified by genome wide computational analysis  
  *Marinee Chuah, Department of Gene Therapy and Regenerative Medicine, Free University of Brussels* |
| 10.15-10.35 | Coffee Break                                                            |
| 10.35-12.20 | Gene therapy on route                                                   |
| 10.35-11.10 | Gene Therapy Approaches in Fanconi anemia: A stem cell disease         
  *Juan Bueren, División de Terapias Innovadoras en el Sistema Hematopoyético, CIEMAT* |
| 11.10-11.45 | Developing successful gene therapies in immunological disorders         
  *Adrian Thrasher, Institute of Child Health, University College London*  |
| 11.45-12.20 | Immune responses following AAV-mediated gene transfer: problems and applications  
  *Anna Salvetti, Centre de Recherche en Cancérologie de Lyon (CRCL), INSERM U1052* |
| 12.30-13.30 | Lunch                                                                   |
| 13.30-15.25 | Gene Based approaches in infection disease (I)                          |
| 13.30-14.05 | Design and application of designer nucleases -- the path to the clinic  
  *Toni Cathomen, Institute for Cell and Gene Therapy & Centre for Chronic Immune Deficiency, University Hospital Freiburg* |
| 14.05-14.40 | Towards HIV Eradication: Excision of proviral DNA by LTR-specific Recombinase  
  *Joachim Hauber, Heinrich-Pette-Institute, - Leibniz Institute for Experimental Virology, Hamburg* |
| 14.40-15.15 | HIV neutralizing antibodies: from bedside to bench and back again?       
  *Ursel Dietrich, Georg-Speyer-Haus, Frankfurt*                           |
| 15.15-15.45 | Coffee Break                                                            |
## PROGRAMME
### THURSDAY 21 APRIL 2016

<table>
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<tr>
<th>Time</th>
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<tr>
<td>15.45-16.55</td>
<td>Gene Based approaches in infection disease (II)</td>
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</table>
| 15.45-16.20   | Vaccine platform recombinant Measles Virus                                                   
                | *Michael Mühlebach, Paul-Ehrlich-Institut, Langen*                                           |
| 16.20-16.55   | Optimization strategies of cytomegalovirus-based vaccine vectors                             
                | *Luka Cicin-Sain, Helmholtz Center for Infection Research, Braunschweig*                     |
| 16.55-17.55   | Immunotherapy                                                                                 |
| 16.55-17.30   | Cell immunotherapy after stem cell transplantation                                           
                | *Renata Stripecke, Department of Hematology, Hemostasis, Oncology and Stem Cell Transplantation, Hannover Medical School* |
| 17.30-18.05   | Designer T cells for immunotherapy of cancer                                                 
                | *Wolfgang Uckert, Institute of Biology, Max-Delbrück-Center for Molecular Medicine, Berlin* |
| 18.10-19.10   | Dinner                                                                                       |
| 19.10         | Find what is missing...                                                                       |
# Programme

**Friday 22 April 2016**

<table>
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<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speaker(s)</th>
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<tbody>
<tr>
<td>9.00-11.20</td>
<td>Targets in Gene and Cell Therapy</td>
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<tr>
<td>9.00-9.35</td>
<td>Manipulation of the vascular system using genes and RNA</td>
<td>Andy Baker, Centre for Cardiovascular Science, University of Edinburgh</td>
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<tr>
<td>9.35-10.10</td>
<td>Can gene therapy help curing Hepatitis B?</td>
<td>Ulrike Protzer, Institute of Virology, Technical University of Munich/ Helmholtz Zentrum München, Munich, Germany</td>
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<tr>
<td>10.10-10.45</td>
<td>iPS-derived cells for metabolic liver disease research</td>
<td>Tobias Cantz, Department of Gastroenterology, Hepatology and Endocrinology, Hannover Medical School</td>
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<tr>
<td>10.45-11.20</td>
<td>Direct reprogramming of hepatic myofibroblasts into hepatocytes in vivo relieves liver fibrosis</td>
<td>Michael Ott, Twincore, Centre for Experimental and Clinical Infection Research, Hannover</td>
</tr>
<tr>
<td>11.20-12.00</td>
<td>Farewell</td>
<td>Wolfgang Uckert, Institute of Biology, Max-Delbrück-Center for Molecular Medicine, Berlin</td>
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<td>German Center for Infection Research Reinhold Schmidt, Clinic for Clinical Immunology, Hannover Medical School</td>
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</tbody>
</table>
SPRING SCHOOL EVALUATION

We do hope you have enjoyed the ESGCT Spring School 2016. We really value your feedback about all aspects of the meeting. 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 Spring School. Once you have completed the survey, you will receive your Certificate of Attendance by email within the following 24 hours.
The European Society of Gene and Cell Therapy (ESGCT), Europe’s only non-profit organisation committed to supporting the advance of basic and translational research and clinical applications of gene and cell therapy.

ESGCT membership offers:

- Congress discount greater than membership fee
- Information about grant opportunities in Europe
  - Instant access to a worldwide network
  - Access to members’ only area of the website
- Online subscription to *Human Gene Therapy* and *HGT Methods*

Nurturing innovation • Developing talent • Collaboration
Advocacy • Public engagement • Global knowledge exchange

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