



EUROPEAN SOCIETY OF
GENE & CELL THERAPY



Sociedad Española
Terapia Génica y Celular

European Society of Gene and Cell Therapy

Spring School 2017



5-7 April 2017

GENYO-Facultad de Medicina-Universidad de Granada,
Granada, Spain

www.esgct.eu



andalusian initiative
for advanced therapies



PRIZER-UNIVERSIDAD DE GRANADA-JUNTA
DE ANDALUCÍA CENTRE FOR GENOMICS
AND ONCOLOGICAL RESEARCH



Universidad de Granada
Facultad de Medicina

Welcome to....

Spring School ESGCT 2017

Venue

1: Nueva Facultad de Medicina
Building PARANINFO
Avenida del Conocimiento s/n.
C.P. 18016. Granada. Spain.

Tel. +34 958 535 050
Email: info@ptsgranada.com
www.ptsgranada.com

2: GENyO
P. T. Ciencias de la Salud
Avda. de la Ilustración 114
18016 Granada, Spain
Tel. +34 958 715 500
Fax. +34 958 637 071

Registration & Information Desk

For registration and information
regarding the Spring School:
Wednesday 5 April 09.00–19.30
Thursday 6 April 08.30–19.30
Friday 7 April 08.30–17.00

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

Tele-Radio-Taxi: +34 958 280 654
Taxi Genil: +34 958 13 23 23

Buses

Stop: La Biblioteca de la Facultad de
Medicina
Bus nos.: SN4 (from city centre), U3

For further information please check the
public transport website:
www.transportesrober.com/transporte/lineas.htm

For more information about visiting
Granada see
<http://en.granadatur.com/>

Getting social!

Follow our official channels:



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www.twitter.com/esgct



www.instagram.com/esgct

Speaker hotel information

Melia Granada
Calle Ángel Ganivet, 7, 18009, Granada
Tel: +34 958 227 400
Email: melia.granada@melia.com
<http://goo.gl/FyJ1Dk>

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



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With thanks to our generous sponsors



WELCOME ADDRESS

On behalf of the European and the Spanish Societies of Gene and Cell Therapy (ESGCT and SETGyC) and the local organising institutions, the Pfizer-Universidad de Granada-Junta de Andalucía Centre for Genomic and Oncological Research (GENYO) and the Universidad de Granada Medical School, it is a great pleasure to welcome you to the second ESGCT Spring School. During these forthcoming three days, international experts on gene and cell therapy will do their best to guide you in a learning journey through gene editing, stem cells, vectors, and diseases, knowing that good students are the best insurance for the progress of their endeavours. Feel free to challenge concepts and ideas, to ask for the details, to interact as much as possible with speakers and other students. It is important to learn what works, but even more to learn what doesn't work, and to acquire the skills to tackle the hurdles. Granada is the perfect setting for inspiration and fun. We wish you will get some of each.

Thanks a lot for coming. Enjoy!



A purple ink signature of Dr. Ramon Alemany.

Dr Ramon Alemany
SETGyC President



A purple ink signature of Dr. Francisco Martin Molina.

Dr Francisco Martin Molina
Local Organising Committee President



A blue ink signature of Prof. Dr. Hildegard Büning.

Prof. Dr. Hildegard Büning
ESGCT Vice-President



ABOUT THE SPRING SCHOOL

The Spring School is an intensive three day lecture course initiated by the ESGCT and in particular dedicated to the training of students and post-docs.

The Spring School is organised by:
Hildegard Büning, ESGCT; DGGT, University of Cologne, DZIF, University Hospital Cologne, Hannover Medical School.

with the help of:

Local Organisers

Francisco Martin, GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia

Marién Cobo, GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia

Pilar Muñoz, GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia

Karim Benabdellah, GENyO: Fundación Progreso y Salud, Sevilla

M José Mora, GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia

SETGyC Organisers

Ramón Alemany, Catalan Institute of Oncology, Barcelona

Gloria Gonzalez-Aseguinolaza, FIMA, Pamplona

Guillermo Güenechea, CIEMAT/CIBERER/IIS-FJD, Madrid



Meeting Organisers:

WATS.ON Ltd

Renée Watson

Gaëlle Jamar

Vanessa Sampson

Emma Clare

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ASSOCIATION MANAGEMENT

With special thanks to

La Fundación Progreso y Salud (FPS) y a la Consejería de Salud de la Junta de Andalucía.



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

**25th Anniversary Meeting
of the ESGCT
in collaboration with the DG-GT
17–20 October 2017 | Berlin**



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 Diemmler, Giuliana Ferrari, Keith Joung, Juergen Knoblich, Andras Nagy, Adrian Thrasher

Parallel speakers include:

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

Plenary sessions on:

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

Parallel sessions on:

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

For updates and registration information see

www.esgct.eu • www.dg-gt.de

PARTNERS

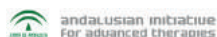
PRINCIPAL PARTNERS



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 Spanish Society of Gene and Cell Therapy (SETGyC) is a non-profit organisation representing scientists and health professionals interested in these innovative therapies. Through its activities, the SETGyC hopes to support basic and translational research aimed at the advancement of gene and cell therapy in our country by promoting the incorporation of the latest international developments in the field. The SETGyC aims to provide a link between researchers, health professionals, regulators and Spanish society to better understand the current state of knowledge and application of gene and cell therapy.
www.setgyc.es



The Andalusian Initiative for Advanced Therapies (IATA) has become a key player of the Andalusian Public Healthcare System. The principal mission of IATA is to foster research in the field of advanced therapies to develop safe and efficient new treatments to offer to the population. The IATA must, therefore, identify, organise and provide the necessary support for the maximum development of multidisciplinary research in the field of advanced therapies in Andalusia. This will require facilitating the training of technologists, basic and clinical researchers, fostering translational research in this field and promoting the generation of a business structure beneficial to such research, which will ultimately provide a source of wealth for the region and will enable the potential benefits of advanced therapies to be passed on to the population in as short a time frame as possible.
<http://www.juntadeandalucia.es/terapiasavanzadas/index.php/es/>

PRINCIPAL PARTNERS



GENyO, Pfizer-Universidad de Granada-Junta de Andalucía Centre for Genomics and Oncological Research, is a mixed centre with stakes held by the Regional Government of Andalucía, the University of Granada and the pharmaceutical company Pfizer. This centre has been devised as a space for excellence research in genomic medicine, focussing on the comprehensive study and understanding of the genetic basis of human diseases in general, placing special emphasis on cancer and genetic disorders related to inheritance. This centre is the benchmark centre of the Andalusian Programme for Research in Clinical Genetics and Genomic Medicine, a programme which, together with those of Cell Therapy and Regenerative Medicine, and Nanomedicine have the main objective of supporting and fostering translational research in Advanced Therapies.

<http://www.genyo.es/en/>



The Universidad de Granada, founded in 1531, continues a long teaching tradition, the roots of which can be traced back to the madrasahs of the last Nasrid Kingdom. Over 60,000 undergraduate and postgraduate students study at the UGR, with another 20,000 students taking additional courses, language courses, summer courses etc. The University employs 3650 lecturers and over 2000 administration, technical and maintenance staff. In Granada, there are four University Campuses, as well as the “Campus Centro”, in which all the centres spread throughout the historic part of the city are brought together. The UGR’s policy of using buildings of historical and cultural value has enriched its heritage, as well as promoting the restoration and maintenance of these buildings.

<http://www.ugr.es/en/pages/universidad/granada>

PARTNERS AND SUPPORTERS



PharmaCell is a leading contract manufacturing organisation for cellular therapies and regenerative medicine in Europe. With a proven track record, PharmaCell covers the full range of manufacturing services. Starting from process development and Clinical Trial Manufacturing from Phase I to Phase III. PharmaCell is also equipped to support and manufacture commercial cell therapy products for the European market.

<http://pharmacell.nl/>



Amarna Therapeutics is a biotech company with a main office in Leiden (The Netherlands) and with a research branch in Sevilla (Spain). Amarna is focused in the application of the SV40-derived viral vectors for the treatment of different indications. In collaboration with several research groups, Amarna is conducting proof of concept studies currently uncured diseases in the fields of ophthalmology, autoimmunity and neurodegeneration (Spain), diabetes (Spain and Austria) and hemophilia (Mexico). Amarna has developed a proprietary platform in Vero cells named SVac, to produce non-replicative SV40 vectors devoid of residual wild type particles. These cells are able to produce high quality viral vector preparations suitable for clinical applications.

<http://www.amarnatherapeutics.com/inbrief/>



Fanconi anemia (FA) is a rare inherited syndrome characterised by the early development of bone marrow failure and increasing predisposition to cancer with age. EuroFancoLen innovative approach is to develop for the first time an efficient and safe gene therapy of FA based on two recent innovations:

- 1) Discovery of potent HSC mobilisers, such as plerixafor Ans
- 2) Development of a new lentiviral vector by members of this Consortium, designed as Orphan Drug by the European Commission in December 2010:

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

www.fanconi.org.uk

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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



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/>



Our mission is to be a centre where collaboration and cooperation between biomedical and clinical research groups are prioritised and furthered, in which special emphasis is placed on aspects of genetic, molecular, biochemical and cell research of rare, genetic or acquired diseases. The aim is to improve our knowledge on epidemiology, the causes and mechanisms of rare diseases. This research is the basis for providing new tools for diagnosis and therapy of rare diseases, backing translational research or transfer research between the scientific medium of the laboratory and the clinical medium of healthcare centres.
www.ciberer.es



The Spanish Fanconi Anemia Association, was born with the aim of improving the support to patients and their families, to promote research on the causes of the disease and new procedures to improve its diagnosis and treatment, and finally to promote awareness of it among professionals, health authorities and society. Among other actions, the Foundation intends to:

- *Be a reference for parents*
- *Promote the development of scientific research projects*
- *Provide support and education to patients and their families*
- *Contribute to the knowledge of the disease*
- *To make visible the problems of patients with Fanconi Anemia*

www.asoc-anemiafanconi.es

PARTNERS AND SUPPORTERS



Human Gene Therapy is the premier journal covering all aspects of human gene therapy, including DNA, RNA, and cell therapies. Human Gene Therapy (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.

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SIRION Biotech is a world leader for innovating virus-based technologies and provides custom services to academic and industrial partners worldwide. SIRION's focus on transduction technologies mastering custom optimization of all 3 major virus types that are used regularly for genetic modification of mammalian cells. SIRION Biotech is globally active, with a strong customer base in cancer research, neurosciences, regenerative medicines, gene therapy, CAR-T cell development and new vaccination methods.

<http://www.sirion-biotech.com/>



Labclinics coordinate the marketing and distribution of equipment and laboratory equipment for research and clinical diagnostics in Spain and Portugal. Our commitment is to provide the best service to the scientific community and biotechnology industry in the peninsula thanks to our sales team, after sales service and our wide range of products, which allows us to supply multiple areas and disciplines of biomedical research and biotechnology production

<http://www.labclinics.com/en/>



ESGCT & BSGCT Spring School 2018

**We look forward to
welcoming you to London.**

Further details available soon.

EUROPEAN SOCIETY OF
GENE & CELL THERAPY



British
Society
for
Gene &
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Therapy

www.ESGCT.eu www.BSGCT.science

PROGRAMME

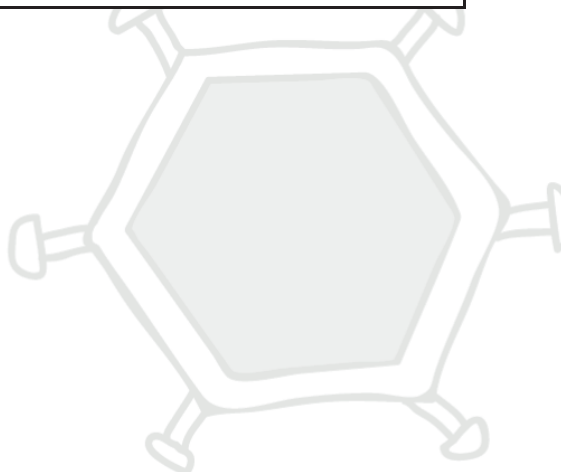
WEDNESDAY 5 APRIL 2017

11:00-13:00	<p>Retos y esperanzas de las terapias avanzadas En español para publico general</p> <p>Terapia celular: una nueva forma de hacer medicina <i>Per Anderson, GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia</i></p> <p>La Terapia génica llega a la clínica <i>Francisco Martín, GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia</i></p> <p>Ingeniería genética: Terapia génica 2.0 <i>Karim Benabdellah, GENyO: Fundación Progreso y Salud, Sevilla</i></p> <p>Situación de las terapias avanzadas en Andalucía <i>Natividad Cuende, Iniciativa Andaluza en Terapias Avanzadas, Sevilla</i></p>
14:00-14:15	<p>Welcome Address <i>Robin Ali, Institute of Ophthalmology, University College London</i></p>
14:15-15:15	<p>Keynote Speaker</p> <p>The origin of CRISPR-Cas9 technology <i>Francis Mojica, University of Alicante</i></p>
15:15-17:15	<p>Gene and cell therapy tools I: How to modify cells <i>in vivo</i> or in cell culture <i>Chair: Miguel García-Toscano</i></p> <p>Adeno-associated vectors and friends – a brief overview on non-integrative viral vectors <i>Hildegard Büning, Institute of Experimental Hematology, Hannover Medical School</i></p> <p>Integrative viral vectors <i>Guillermo Güenechea, CIEMAT / CIBERER / IIS-Fundación Jiménez Díaz, Madrid</i></p> <p>Engineering the genome with the <i>Sleeping Beauty</i> transposon system <i>Zoltan Ivics, Paul-Ehrlich-Institut, Langen</i></p>
17:15-17:45	Coffee Break
17:45-19:15	<p>Gene and cell therapy tools part II: upcoming technologies and safety consideration <i>Chair: Karim Benabdellah</i></p> <p>Gene editing <i>Paula Rio, CIEMAT / CIBERER / IIS-Fundación Jiménez Díaz, Madrid</i></p> <p>Tissue engineering: a fast changing field <i>Ander Izeta, Instituto Biodonostia, San Sebastian</i></p>
19:30	Welcome Reception in Paraninfo

PROGRAMME

THURSDAY 6 APRIL 2017

9:00-10:20	Modelling human disease <i>Chair: Pedro Real</i>
	Cellular and animal models of neurodegenerative diseases: Focus on Parkinson's disease <i>Jose Luis Labandeira-Garcia, Universidad de Santiago de Compostela</i> Human pluripotent stem cells: iPSCs and hESCs <i>Pedro Real, GENyO - Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia</i>
10.20-11.40	Gene and cell therapy strategies: Blood disorders <i>Chair: Pilar Muñoz</i>
	Haematological disorders <i>Anne Galy, Généthon, Evry</i> Bone marrow failure syndromes <i>Juan Bueren, CIEMAT/CIBERER, Madrid</i>
11:40-12:10	Coffee Break
12:10-14:10	Gene and cell therapy strategies: Neurodegenerative diseases <i>Chair: Rosario Sanchez Pernaute</i>
	Gene therapy of CNS disorders <i>Nathalie Cartier, INSERM UMR1169, Université Paris-Sud; CEA, DSV, FBM, MIRCen, Fontenay-aux-Roses</i> Cell therapy of Parkinson disease <i>Juan José Toledo-Aral, SETGyC/Instituto de Biomedicina de Sevilla</i> Gene therapy of retinal degeneration <i>Robin Ali, Institute of Ophthalmology, University College London</i>
14:10-15:10	Lunch



PROGRAMME

THURSDAY 6 APRIL 2017

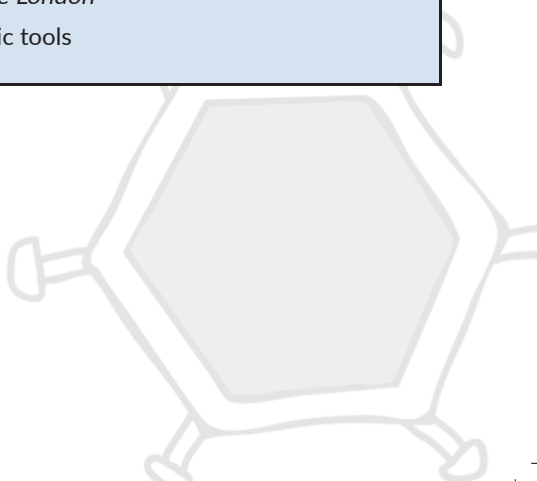
15:10-16:30	Gene and cell therapy strategies: Inflammatory and cardiovascular diseases <i>Chair: Mario Delgado</i>
	Cardiovascular cell therapy <i>Pilar Sepulveda, Instituto de Investigación Sanitaria La Fe</i> Inflammatory diseases <i>Mario Delgado, Institute of Parasitology and Biomedicine IPBLN-CSIC, Granada</i>
16:30-17:00	Coffee Break
17:00-18:20	Gene and cell therapy strategies: skin and infectious diseases <i>Chair: Daniel Bachiller</i>
	Skin disorders <i>Marcela del Rio, CIEMAT / CIBERER / UC3M IIS-Fundación Jiménez Díaz, Madrid</i> Infectious diseases <i>Daniel Bachiller, CSIC, Bunyola</i>
18:20-19:00	Safety considerations of gene therapy strategies <i>Chair: Guillermo Güenechea</i>
	Safety and efficacy issues of gene therapy vectors: The pharmacodynamics of gene therapy <i>Christof von Kalle, CT DKFZ, Heidelberg</i>
20:30	'Meet the Experts' Dinner At Carmen de los Chapiteles



PROGRAMME

FRIDAY 7 APRIL 2017

09:00-11:00	Gene and cell therapy strategies: Metabolic and lysosome storage disorders <i>Chair: Gloria Gonzalez Aseguinolaza</i>
	Gene therapy for liver inherited diseases <i>Gloria Gonzalez Aseguinolaza, FIMA, Pamplona</i> Lysosome storage disorders <i>Nicolina Cristina Sorrentino, Fondazione Telethon - TIGEM, Pozzuoli</i> Towards a gene therapy for neurological and somatic mucopolysaccharidosis <i>Fatima Bosch, Universidad Autonoma de Barcelona</i>
11:00-11:30	Coffee Break
11:30-13:30	Gene and cell therapy strategies: Cancer <i>Chair: Francisco Martin</i>
	Engineered T cells for cancer treatment <i>Chiara Bonini, San Raffaele Scientific Institute, Milan</i> Virotherapy, basic concepts <i>Ramón Alemany, Catalan Institute of Oncology, Barcelona</i> Virotherapy, clinical applications <i>Manuel Ramirez, Hospital Universitario Niño Jesús, Madrid</i>
13:30-14:30	Lunch
14:30-16:30	Breaking towards gene and cell therapy <i>Chair: Natividad Cuende</i>
	Translating gene therapy tools to clinical applications in inherited immunodeficiencies <i>Adrian Thrasher, University College London</i> Gene modified HSCs as therapeutic tools <i>Luigi Naldini, SR-Tiget, Milan</i>



Meet the Experts Dinner

Exchange opinions and meet internationally acclaimed scientists in a relaxed setting.

Enjoy breathtaking views from the spectacular venue of Carmen de los Chapiteles

Thursday 6 April, 9pm

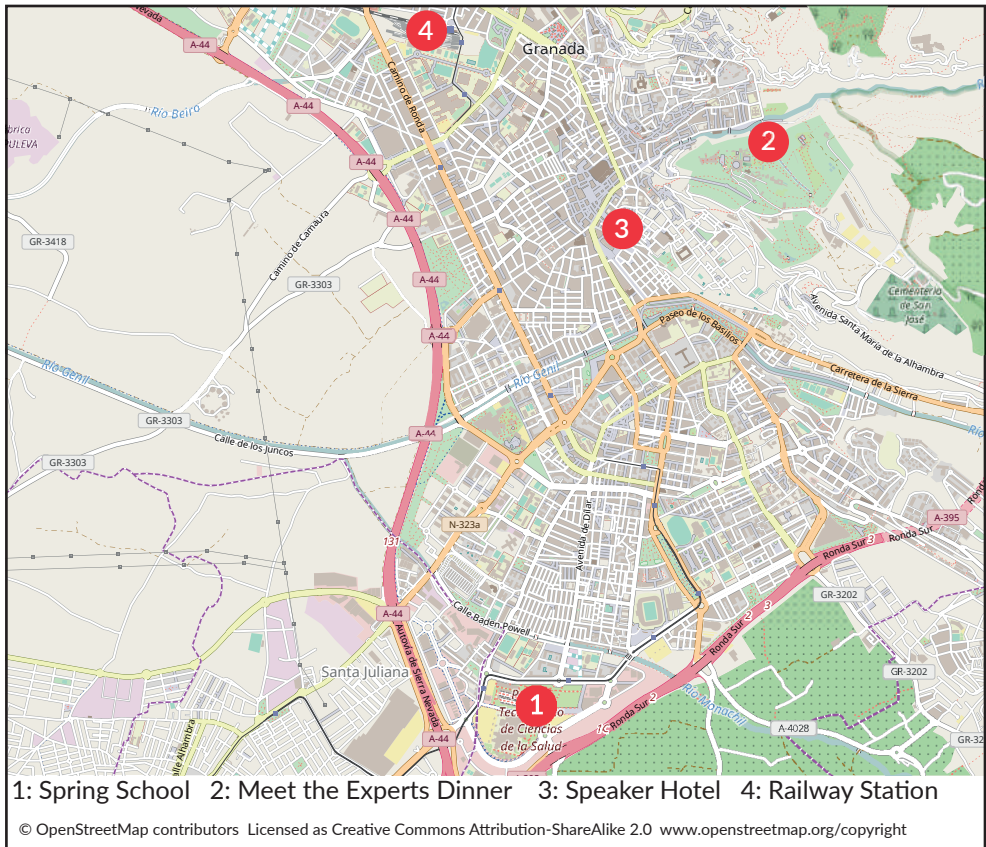


Take bus SN4 to Catedral and walk for 15 minutes along Calle Reyes Católicos. Turn right along Cuesta del Rey Chico and left along Camino Fuente del Avellano to reach Carmen de los Chapiteles.

Attendees are required to have registered in advance.



MAP OF GRANADA



SPRING SCHOOL EVALUATION

We do hope you have enjoyed the ESGCT Spring School 2017. 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 once these are sent out the week of the 24th April (which gives you two weeks to complete the survey). Certificates will NOT be sent out until after this date.



SAVE THE DATE

Spanish Society for Gene and Cell Therapy

Biennial Congress

14-16 March 2018

Hotel Barcelo Illetas Albatros, Palma de Mallorca



www.setgyc.es