



# Spanish Society of Gene and Cell Therapy

# 9<sup>th</sup> Biennial Congress Programme and Information Book

14-16 March 2018

Barceló Illetas Albatros, Palma de Mallorca, Spain



# Palma de Mallorca

#### The Barceló Illetas Albatros

The Congress takes place in the spectacular Barceló Illetas Albatros hotel, situated on the outskirts of Palma de Mallorca.

Located on the seafront in an idyllic cove of the Bay of Palma, you can enjoy sunshine and sea views in this tranquil Mediterranean setting.

PS de Illetas, 15 07181 Illetas, Calviá

Tel. +34 971 402 211

E-mail: illetasalbatros@barcelo.com Website: https://goo.gl/7Wuiwf

# Registration & Information Desk

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

Wednesday 14 March 08.00–21.30 Thursday 15 March 09.00–20.00 Friday 16 March 08.30–14.30

#### In case of emergency, contact:

Gaëlle Jamar, Event Manager Tel: +44 7766 475379 Email: office@setgyc.es

#### **Taxis**

Taxis are available from taxi ranks outside the airport and close to the hotel. Other taxi providers include:

Taxi Calvià +34 971 13 47 00 Taxi PMI +34 627 017 681

#### Buses

The venue is located on bus route LINEA 3. From the airport, take LINEA 1 to PLAZA ESPAÑA and change for LINEA 3.

Visit www.emtpalma.cat for information and timetables.

#### **Getting social at SETGYC!**

Follow our official social media channels:



www.facebook.com/setgyc



www.twitter.com/setgyc



www.instagram.com/setgyc

Make sure you use the official hashtag #SETGYC2018 in your posts, and check out the latest Congress news and updates!

For more information about visiting Palma de Mallorca see www.visitpalma.com

Book design based on Congress programmes produced by Catherine Charnock Creative: www.catherinecharnock.co.uk

#### **Gold Partners**









#### Silver Partners

















#### **Bronze Partners**







# Supported By:



# **WELCOME ADDRESS**

On behalf of the organising committee and the Spanish Society of Gene and Cell Therapy (SETGYC), it is a great pleasure to welcome you to the 9th Biennial Congress of the Spanish Society of Gene and Cell Therapy held in Palma de Mallorca.

Gene and cell therapy is revolutionising medicine. Since our previous congress in November 2015 held in San Sebastian, our field has matured to become a therapeutic reality for several diseases. New cell therapies have been approved for conditions like graft vs host disease and cartilage defects, adding to a long list of previously approved stem and non-stem cell therapies for tissue injuries. Gene therapy has blossomed with new products such as Spinraza for spinal muscular atrophy, Strimvelis for adenosine deaminase deficiency, Imlygic for melanoma, Kymriad for acute lymphoblastic leukemia, Yescarta

for non-Hodgkin lymphoma, and Luxturna for retinal dystrophy. Clinical success has attracted big pharma to the field, and new challenges for product development marketing have emerged.

Spain's outstanding record in transplantation medicine and its unique universal public health system offer clear opportunities to our field, as reflected by our leading position in Europe in clinical trials involving somatic cell therapy medicinal products and tissue engineered products.

In this meeting, we aim at improving the quality of our work, fostering collaborations, and especially motivating young students to join this exciting and promising field. For this purpose, we will run educational sessions on broad topics such as viral vectors and stem cells, as well as on specific hot areas like gene editing and iPS cells. Plenary

sessions will host internationally recognised researchers with special attention to scientific leaders from our country. Two parallel sessions will cover specific topics as well as selected oral presentations among the submitted abstracts.

We hope you will enjoy the science and social activities at this beachfront Barceló Illetas Albatros hotel near the beautiful city of Palma de Mallorca.



Ramón Alemany SETGYC President



Daniel Bachiller Organising Committee President

Land Rolling

#### SETGYC BOARDS AND ORGANISING COMMITTEE

#### SETGYC Board

Ramón Alemany (President) Instituto Catalán de Oncología/ Instituto de Investigación Biomédica de

Bellvitge, Barcelona

Angel Raya (Vice President) ICREA, Institut de Bioenginyeria de Catalunya (IBEC). Barcelona

Guillermo Güenechea Amurrio (Treasurer) CIEMAT, CIBERER, IIS-FJD, Madrid

Gloria Gonzalez-Aseguinolaza (Secretary)

Centro de Investigación Médica Aplicada

(CIMA), Pamplona

Ander Izeta

Instituto Biodonostia, San Sebastián

José Luis Labandeira

Centro de Investigación en Medicina

Molecular (CIMUS), Santiago de

Compostela

Manuel Ramírez

Hospital Universitario Infantil Niño

Jesús, Madrid

Paula Rio

CIEMAT/CIBERER, IIS-FJD, Madrid

Pilar Sepúlveda

Instituto de Investigación Sanitaria La

Fe, Valencia

Juan José Toledo-Aral Universidad de Sevilla

# **Local Organising Committee**

Daniel Bachiller
IMEDEA, CSIS, ESPORLES, Mallorca

#### **ORGANISERS**

WATS.ON Ltd.

Renée Watson Gaëlle Jamar Vanessa Sampson Emma Clare Metta Pratt Christina Slater

www.wats-on.co.uk



#### **AWARDS**

#### **Travel Grants**

Congratulations to the following researchers on being awarded Travel Grants. The grants are awarded to the 6 highest-scored abstracts submitted by PhD students and first post-doctoral Congress delegates.



#### Jose Bonafont

Carlos III University (UC3M), Madrid

OR023: CRISPR/Cas9-based gene editing approaches for the efficient correction of recessive dystrophic epidermolysis bullosa patient derived-epidermal stem cells

#### Clara Nicolas

Mayo Clinic, Rochester, MN

OR014: Curing disease before birth: *in utero* gene therapy for the treatment of hereditary tyrosinemia type 1

#### Júlia Vallverdú

IDIBAPS, Barcelona

OR012: Generation of hepatic stellate cells by directed differentiation of human pluripotent stem cells

#### María Rosario Hervás

CIEMAT/CIBERER/IISFJD. Madrid

OR028: Enhanced anti-inflammatory potential of mesenchymal stromal cells mediated by the ectopic transient expression of CXCR4 and IL10

#### Belén García-Lareu

Institut de Neurociencies (INc) UAB; CIBERNED, Barcelona

OR024: Specific expression of GDNF in muscles as gene therapy strategy for ALS

#### Matteo Libero Baroni

Josep Carreras Leukemia Research Institute, Barcelona

OR029: Preclinical optimisation of CAR CD123-expessing T-cells for acute myeloid leukaemia

### **ESGCT Young Investigator Award**



The ESGCT Young Investigator Award is awarded in recognition of valuable contributions to the field of gene and cell therapy. The award winner will be announced during the Congress.

# Viral Vectors : finding the good in everyone

A global and integrated offer from vector development to product release



# New commercial facility for Viral Vectors in Belgium:

- > Up to 2,000L scale
- > Ready for production in 2019



# **SAVE THE DATE!**

# ESGCT & SETGYC CONGRESS 2019

21 October - 25 October CCIB, Barcelona, Spain



www.esgct.eu





#### **GOLD PARTNERS**



Rocket Pharma is a clinical-stage gene therapy company with a platform approach for the treatment of patients with high-unmet need rare diseases. We leverage lentiviral and AAV transduction strategies towards first-in-class programs. Hallmarks of Rocket's vision include: 1) a high threshold for the selection of quality programs, 2) leverage of deep industry know-how through a world-class team (Novartis Cell & Gene, Bluebird, BMS, others) and manufacturing partnerships (MolMed, PCT, others) and 3) a laser focus on optimizing gene therapy product parameters through a seasoned scientific approach that de-risks programs as they enter clinical trials.

www.rocketpharma.com



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

www.red-tercel.com



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

#### **GOLD PARTNERS**



Vivet Therapeutics is a gene therapy biotech company with headquarters in Paris, France, dedicated to the research, development and future commercialization of gene therapy products for inherited liver disorders with high medical need. Recently named as Fierce 15 biotech company, Vivet is committed to make a significant difference for patients facing rare metabolic diseases, with Wilson's disease being our lead indication. Vivet has a strong presence in Spain through its research subsidiary and its strategic collaboration with Fundación para la Investigación Médica Aplicada (FIMA) at the Centro de Investigación Medica Aplicada (CIMA, University of Navarra, Spain), a leading research organization in the field of Gene Therapy. Vivet also has licensed the use of novel proprietary AAV vector gene therapy technology to treat metabolic diseases and certain patent-protected Anc80 AAV gene therapy vectors from Massachusetts Eye and Ear (MEE), a teaching hospital of Harvard Medical School, Boston. Anc80 is a next-generation gene therapy technology designed to increase gene expression levels in the liver.

www.vivet-therapeutics.com/en

#### SILVER PARTNERS



Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include ABO-102 (AAV-SGSH), an adeno-associated virus (AAV)-based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and EB- 101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB). Abeona is also developing ABO-101 (AAV-NAGLU) for Sanfilippo syndrome type B (MPS IIIB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona is developing its proprietary vector platform, AIM™, for next generation product candidates.

http://abeonatherapeutics.com/

#### SILVER PARTNERS

# **AUDENTES** >

Audentes Therapeutics is a clinical stage biotechnology company focused on developing and commercializing gene therapy products for patients living with serious, life-threatening rare diseases. Audentes is currently developing four product candidates, including AT132 for the treatment of X-Linked Myotubular Myopathy (XLMTM), AT342 for the treatment of Crigler-Najjar Syndrome, AT982 for the treatment of Pompe disease, and AT307 for the treatment of the CASQ2 subtype of Catecholaminergic Polymorphic Ventricular Tachycardia (CASQ2-CPVT). We are a focused, experienced and passionate team committed to forging strong, global relationships with the patient, research and medical communities.

www.audentestx.com/



BD Biosciences is a world leader in bringing innovative diagnostic and research tools to life science researchers, clinical researchers, laboratory professionals and clinicians who are involved in basic research, drug discovery and development, biopharmaceutical production and disease management. The BD Biosciences segment is focused on continually advancing the science and applications associated with cellular analysis and products that help grow living cells and tissue.

Products/Services:

- 1) Fluorescence-activated cell sorters and analysers:
- 2) Monoclonal antibodies and kits for cell analysis;
- 3) Reagent systems for life science research;
- 4) Cell culture media and supplements for biopharmaceutical manufacturing.

www.bdbiosciences.com

# **BIOMARIN**

With six products on the market and a fully-integrated multinational organization in place, BioMarin is providing innovative therapeutics to patients with serious unmet medical needs. The company is also currently conducting a clinical trial of an AAV-based potential gene therapy for hemophilia A.

www.biomarin.com

#### SILVER PARTNERS



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 knowhow, 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



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

www.vcnbiosciences.com



Viralgen vector Core is a new Company focused on GMP Development and Manufacturing of AAV. We are able to produce Research, Preclinical and GMP-grade batches. Our objective is became the CDMO leading in the sector of gene therapy and innovative medicines in Europe and US for developing and manufacturing in order to contribute to the progress in the health and welfare of people.

www.viralgenvc.com

#### SILVER PARTNERS



VIVEbiotech is a GMP Contract Development and Manufacturing Organization (CDMO) specialized in Lentiviral vectors. VIVE is focused on the manufacture of Research, Preclinical toxicology and GMP-grade lentiviral vectors. Sealed as Excellence Centre by European Union, VIVE is currently working with Companies based both in Europe and USA being our main interest adapting to Customer requirements considering also planning and timelines related issues. Additionally, VIVE has developed LENTISOMA's technology, a non-Integrative lentiviral episomal replicative vector whose main advantage is associated to Safety-related aspects. www.vivebiotech.com

#### **BRONZE PARTNERS**



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



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 groundbreaking safety and efficacy patient monitoring studies. www.fanconi.org.uk

#### **BRONZE PARTNERS**



Généthon, created by the AFM Téléthon, has a mission to make innovative gene therapy treatments available to patients affected by rare genetic diseases. To meet this challenge Généthon has assembled the technical and human resources needed to accelerate the medical application of scientific discoveries arising from fundamental research. Strong translational research programmes engage multi-disciplinary teams and are supported by a first-rate technological platform and cGMP facility.

The pipeline of Généthon includes products currently in international clinical trials and at preclinical stages, for immune deficiencies, muscular dystrophies, ocular and liver diseases. These products are developed either with Généthon as sponsor, or in partnership with private companies and academic institutions. www.Généthon.fr

#### SUPPORTED BY:



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





Exclusive academic research collaboration on Gene Therapies for liver indications: design of constructs, animal models, in vitro & in vivo Proof of Concept



Anc80 AAV license for liver metabolic disorders

5 PROGRAMS
innovative preclinical
development for rare
liver metabolic
diseases via academic
collaborations



Viral vector technology improvements (sustainability & immune response)



Manufacturing process optimization GMP production @ CMOs



Building Physicians, Payers & Patients advocacy groups networks in WD, CTLN, PFIC Addressing
SUSTAINED
EXPRESSION AND
IMMUNE RESPONSE
with unique GT platform

NEW LIVER
TARGETING
AAV-ANC80
SEROTYPE

STRONG IP
POSITION
on AAVs and
Wilson's Disease



# **Gala Dinner Evening**

Join us for an evening of Mediterranean cuisine at the Restaurante Caballito de Mar. Situated near the cathedral and Palma's old quarter, take in the culture of Palma in this historic setting.

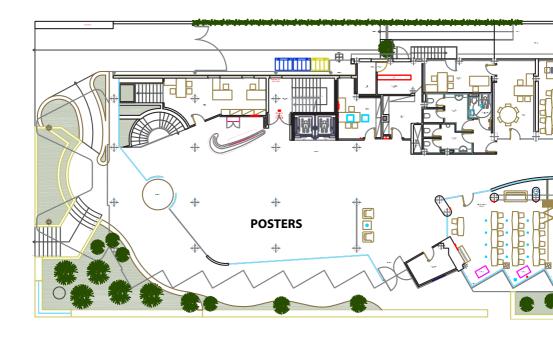
Free of charge, but attendees must register in advance

Restaurante Caballito de Mar, Paseig de Sagrera Thursday 15 March, 9pm

Coaches to the dinner venue will be leaving from the Barceló Illetas Albatros hotel at 8.30pm



# **EXHIBITORS**







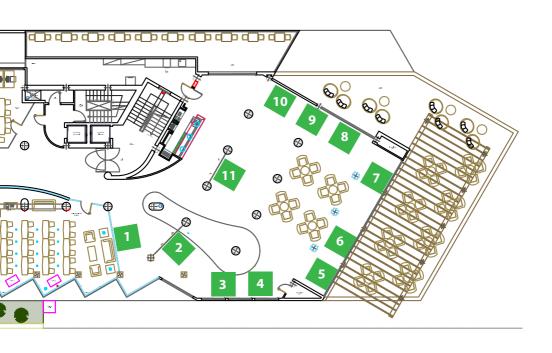






























# biotechne

BOOTH 1: Bio-Techne brings together some of the most referenced brands in life science - R&D Systems, Novus Biologicals, Tocris Bioscience, and ProteinSimple providing innovative, high-quality research tools, including:

- Bioactive proteins R&D Systems premiere bioactive proteins
- Application-qualified Antibodies a diverse and extensive analyte selection from Novus and R&D Systems
- Immunoassays Legendary R&D Systems Quantikine ELISAs, our huge selection of Luminex Assays and cost effective Proteome Profiler Arrays.
- High quality small molecules a unique collection of over 3,500 Tocris reagents

Together we are Bio-Techne. Find out how we can be your partner and help you attain your research goals by visiting our stand. www.bio-techne.com



BOOTH 2: Abeona Therapeutics: See Partner Listings for details https://abeonatherapeutics.com/



BOOTH 3: ChemoMetec develops, manufactures and sells high quality automated Image Cytometer's within cell counters, which are the only ones on the market that 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 4:** EuroClone®: See Partner Listings for details www.euroclonegroup.it



BOOTH 5: VIVEbiotech: See Partner Listings for details www.vivebiotech.com





BOOTH 6: Maco Pharma is one of the key players in the medical device industry for blood transfusion, solution bags and biotherapy. Their growth has always been driven by major innovations. These innovations are the result of substantial investments in research and development, as well as cooperation with customers and partners: blood centres, research laboratories, hospitals. The Biotherapy range of products provides solutions for every step of the cellular therapy process from collection, processing, expansion and cryopreservation of stem cells, up to the treatment and transplantation of cells or organs to patients. To cope with this challenge Maco Pharma developed a secure cell culture concept with xeno-free culture media and a full range of human platelet lysate called MultiPL': an efficient, safe and standardised supplement to expand multiple cell types. Our MultiPL' range can be used both for research and clinical use www.macopharma.com



BOOTH 7: Delphi Genetics develops innovative technologies and provides services in (i) production of plasmid DNA (pDNA) and recombinant proteins and (ii) antibody development.

The Staby® technology was developed to replace the antibioticresistance gene by a selection system that uses two natural bacterial genes. The technology is already used successfully for industrial productions in E. coli of recombinant proteins and pDNA to achieve higher yields using antibiotics-free media. The Staby® technology meets the regulatory guidelines for the use of plasmid DNA in gene therapy (naked plasmid DNA and for the production of recombinant viruses). Additionally, the technology guarantees plasmid stability during production scale-up and increases production efficiency.

The company developed a complete single-use process for GMP-compliant productions of plasmid DNA and collaborates on projects at all stages of development from R&D to clinical phases with or without technology. The company has its own facilities for productions in R&D, High Quality and GMP.

www.delphigenetics.com

#### **EXHIBITORS**



BOOTH 8: PeproTech creates the building blocks of your life science research by manufacturing high-quality products that advance scientific discovery and human health. Since 1988, we have grown into a global enterprise with state-of-the-art manufacturing facilities in the US, and offices around the world.

With over 2,000 products PeproTech has developed and refined innovative protocols to ensure quality, reliability and consistency. Our mission is to provide the highest quality products and premium support that address the needs and demands of today's scientists and researchers. We pride ourselves on being a trusted partner within the scientific community.

#### Our products:

- Comprehensive line of Cytokines and Antibodies
- GMP Cytokines for Cell, Gene and Tissue Therapy
- Animal-Free Cytokines
- ELISA Development Kits
- Cell Culture Media Kits / Supplements www.peprotech.com



BOOTH 9: As part of Merck KGaA, Darmstadt, Germany, BioReliance® Services is a key component of the life science services portfolio and the leading provider of contract services in the biopharmaceutical industry. BioReliance's comprehensive and integrated services support every phase of the testing, development and manufacturing process. With locations worldwide, BioReliance offers more than 1,000 tests and complementary services related to biologics safety testing and specialized toxicology. Our clients include the world's top producers of traditional pharmaceuticals, medical devices and chemicals, as well as biopharmaceuticals http://www.bioreliance.com



BOOTH 10: STEMCELL Technologies is a leader in the development of specialized cell isolation products, cell culture media, and supporting reagents for cellular therapy research, including mesenchymal stem cell and T cell therapy research. Our tools also include reagents for maintenance and differentiation of stem and progenitor cells, culture of organoids from various tissues. Driven by science and a passion for quality, STEMCELL Technologies delivers over 1500 products to more than 70 countries worldwide. Learn more at www.stemcell.com.

www.stemcell.com



BOOTH 11: Miltenyi Biotec is a global provider of products and services that advance biomedical research and cellular therapy. Our innovative tools support research at every level, from basic research to translational research to clinical application. This integrated portfolio enables scientists and clinicians to obtain, analyze, and utilize the cell. Our technologies cover techniques of sample preparation, cell isolation, cell sorting, flow cytometry, cell culture, molecular analysis, and preclinical imaging. Our more than 25 years of expertise spans research areas including immunology, stem cell biology, neuroscience, and cancer, and clinical research areas like hematology, graft engineering, and apheresis. In our commitment to the scientific community, we also offer comprehensive scientific support, consultation, and expert training. Today, Miltenyi Biotec has more than 1,400 employees in 25 countries - all dedicated to helping researchers and clinicians around the world make a greater impact on science and health.

www.miltenyibiotec.com

# **PROGRAMME**

|                          | Parallel s               | essions  |
|--------------------------|--------------------------|--|
| 9.00-10.30<br>Sala Vent  |                          | nal 1a - Viral vectors<br>nn Carlos Ramirez, ViveBiotech, San Sebastián-Donostia   |
|                          | INV001                   | Lentiviral vectors<br>Juan Carlos Ramirez, ViveBiotech, San Sebastián-Donostia   |
|                          | INV002                   | Adeno-associated viral vectors: a big challenge for a small virus<br>Eduard Ayuso, <i>University of Nantes</i>   |
|                          | INV003                   | Adenoviral vectors: evolution and current perspectives<br>Ruben Hernandez-Alcoceba, <i>University of Navarra</i> , <i>Pamplona</i>                                 |
|                          | Round ta                 | ble (15 minutes)   |
| 9.00-10.30<br>Sala Mar   |                          | nal 1b - Cellular therapy: TERCEL vier García-Sancho, University of Valladolid   |
|                          | INV004                   | Treatment of osteoarticular diseases with mesenchymal stromal cells Javier García-Sancho, <i>University of Valladolid</i>  |
|                          | INV005                   | Understanding stemness: multipotency vs dedifferentiation, and other confounding factors Ander Izeta, <i>Instituto Biodonostia</i> , <i>San Sebastián-Donostia</i> |
|                          | INV006                   | Cell therapy for Parkinson´s disease: a historical perspective<br>José L. Labandeira-Garcia, CIMUS-IDIS, University of Santiago de<br>Compostela                   |
|                          | Round ta                 | ble (15 minutes)   |
| 10.30-11.00              | Coffee bi                | reak   |
| 11.00-12.30<br>Sala Vent |                          | nal 2a - Gene editing tools<br>s Montoliú, CNB-CSIC & CIBERER-ISCIII, Madrid   |
|                          | INV007                   | Generation of new animal models of rare diseases using CRISPR tools<br>Luis Montoliú, CNB-CSIC & CIBERER-ISCIII, Madrid  |
|                          | INV008                   | CRISPR/Cas9: a revolutionary tool for cancer modelling<br>Raul Torres, CNIO, Madrid; Josep Carreras Leukaemia Research<br>Institute, Barcelona                     |
|                          | INV009                   | Skeletal muscle: CRISPR/Cas9 approaches to study embryonic development and human disease Jaime Carvajal, Centro Andaluz de Biología del Desarrollo, CSIC, Sevilla  |
|                          | Round table (15 minutes) |  |

| 11.00-12.30                             | Educational 2b - Disease modelling using iPSC  |  |
|---|--|--|
| Sala Mar                                | Chair: Angel Raya, CMRB, Barcelona   |  |
|   | INV010 Cell reprogramming for rare disease modelling: perspectives and challenges  Juan Roberto Rodriguez-Madoz, CIMA, Pamplona                            |  |
|   | INVO11 Modelling aortic aneurisms in vitro using hiPSC-derived smooth muscle cells Felipe Serrano, The University of Cambridge                             |  |
|   | INVO12 In vitro disease modeling with iPSC-derived neurons applied for functional phenotypic drug screening Benjamin Bader, Neuroproof, Rostock            |  |
|   | Round table (15 minutes)   |  |
| 12.30-14.00                             | Lunch  |  |
|   | Plenary session  |  |
| <b>14.00-15.15</b> <i>Sala Marivent</i> | Inaugural session Chairs: Ramón Alemany, President of the SETGYC (SETGYC society update); Guillermo Güenechea, CIEMAT/CIBERER/IIS-FJD, Madrid              |  |
|   | INV013 Gene therapy in severe immunodeficiency Adrian Thrasher, University College London  |  |
|   | INV014 Decoding and recoding midbrain dopaminergic neurons for Parkinson's disease cell replacement therapy Ernest Arenas, Karolinska Institute, Stockholm |  |
| 15.15-16.30<br>Sala Marivent            | Plenary session 1<br>Chairs: Ramón Alemany, President of the SETGYC; Guillermo Güenechea,<br>CIEMAT/CIBERER/IIS-FJD, Madrid                                |  |
|   | INVO15 Ex vivo gene therapy of non-conditioned Fanconi anaemia patients Juan Bueren, CIEMAT/CIBERER/IIS-FJD, Madrid  |  |
|   | INV016 Stem cell niche biology in the adult brain<br>Isabel Fariñas, <i>University of Valencia</i>   |  |
| 16.30-17.00                             | Coffee break   |  |

# **PROGRAMME**

|                          | Parallel s | essions   |
|--------------------------|------------|---|
| 17.00-18.30<br>Sala Vent |            | a - Regulatory and manufacturing Ruíz, AEMPS, Madrid  |
|                          | INV017     | Regulatory aspects of ATMP in the EU<br>Sol Ruiz, AEMPS, Madrid   |
|                          | INV018     | Addressing industrialisation and manufacturing hurdles Ricardo Baptista, Cell and Gene Therapy Catapult, London   |
|                          | OR001      | Manufacturing of GMP-grade lentiviral vectors in solid-phase bioreactors: goals, hurdles and challenges Juan Carlos Ramirez, VIVEbiotech, San Sebastián-Donostia  |
|                          | OR002      | Expansion of human natural killer cells used for adoptive immunotherapy in a fully automated system María Vela, Fundación Investigación Hospital La Paz; Hospital La Paz; Centro Nacional de Investigaciones Oncológicas, Madrid                                |
|                          | OR003      | Development of clinical-grade doxycycline-inducible CAR T cells<br>Noelia Maldonado, GENyO- Centro de Genomica e Investigacion<br>Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia;<br>Hospital Reina Sofía-Unidad de terapia celular (Córdoba) |
| 17.00-18.30<br>Sala Mar  | Chair: Me  | b - Organoids and gene transfer models<br>ritxel Huch, Wellcome Trust/Cancer Research UK Gurdon Institute,<br>of Cambridge  |
|                          | INV019     | TALEN-mediated correction of p.F508del and restoration of CFTR function in hiPS-derived intestinal organoids Arne Fleischer, Mediterranean Institute for Advanced Studies (IMEDEA); CSIC, Mallorca  |
|                          | INV020     | Hepatic organoids to model human liver disease<br>Meritxel Huch, Wellcome Trust/Cancer Research UK Gurdon<br>Institute, University of Cambridge   |
|                          | OR004      | Patient-derived pancreatic cancer organoids as a platform for<br>testing oncolytic virotherapy<br>Giulia Raimondi, Institut d'Investigacions Biomèdiques August Pi i<br>Sunyer (IDIBAPS), Barcelona   |
|                          | OR005      | The IS2 element improves transcription efficiency of integration-deficient lentiviral vector (IDLV) episomes María Rocío Martín-Guerra, GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / Junta de Andalucia              |
|                          | OR006      | Disease modeling of hereditary retinal dystrophies using patients' iPSCs Dunja Lukovic, Centro de Investigación Príncipe Felipe, Valencia   |

| 18.30-20.00<br>Sala Vent | Parallel 2a - Gene editing<br>Chair: José Carlos Segovia, CIEMAT/CIBERER/IIS-FJD, Madrid |  |             |
|--------------------------|--|--|-------------|
|                          | INV021   | Gene editing in Fanconi anaemia haematopoietic stem an<br>progenitor cells<br>Paula Río, CIEMAT/CIBERER/IIS-FJD, Madrid  | nd          |
|                          | INV022   | Efficacy and safety of genome editing tools: Wiskott-Aldr<br>syndrome as a model<br>Francisco Martín, <i>Genyo</i> , <i>Granada</i>  | rich        |
|                          | OR007  | Efficient gene editing of human haematopoietic stem cell<br>Francisco José Román-Rodríguez, CIEMAT; CIBERER; IIS-FJ<br>UAM, Madrid   |             |
|                          | OR008  | Modelling genome edition of Wiskott-Aldrich locus: from target cleavage to off-target donor integrations Araceli Aguilar González, GENyO- Centro de Genomica e Investigacion Oncologica: Pfizer / Universidad de Granada / J |             |
|                          | OR009  | CRISPR/Cas-mediated disruption of the glycolate oxidase efficacious and safe treatment for primary hyperoxaluria Nerea Zabaleta, CIMA, Pamplona  |             |
| 18.30-20.00<br>Sala Mar  |  | b - Pluripotent stem cells and cell reprogramming<br>gel Raya, CMRB, Barcelona   | <b>⊘</b> BD |
|                          | INV023   | Novel insights into Parkinson's disease through iPSC-base technology<br>Angel Raya, CMRB, Barcelona  | ed          |
|                          | INV024   | iPSC-based modelling of human genetic and malignant di<br>Pablo Menendez, Josep Carreras Leukaemia Research Instit<br>University of Barcelona  |             |
|                          | OR010  | Generation and validation of fluorescent TH reporter line<br>human iPS cells using CRISPR/Cas9 technology<br>Carles Calatayud Aristoy, Institut de Biomedicina Universita<br>Barcelona                                       |             |
|                          | OR011  | Primordial germ cells differentiation from human induced pluripotent stem cells of infertile men carrying genetic abnormalities  Aurélie Mouka, <i>Université Paris-Saclay</i> , <i>Orsay</i> ; <i>INSERM</i> , 750.         |             |
|                          | OR012  | Generation of hepatic stellate cells by directed differential human pluripotent stem cells Júlia Vallverdú, Institut d'Investigacions Biomèdiques August Sunyer (IDIBAPS), Barcelona   |             |
| 20.00-21.30              | Welcome  | e and poster session 1   |             |

# **PROGRAMME**

|                              | Plenary s  | assians   |                  |
|------------------------------|--|---|------------------|
| 9.00-10.00                   |  |   |                  |
| Sala Marivent                | Plenary session 2<br>Chair: José Luis Labandeira, CIMUS-IDIS, University de Santiago de Compostela |   | Compostela       |
|                              | INV025   | Gene therapy of mucopolysaccharidosis<br>Fátima Bosch, Autonomous University of Barcelona   |                  |
|                              | INV026   | Targeting the stem-cell niche in myeloproliferative no<br>Simón Méndez-Ferrer, WT-MRC, Cambridge Stem Cell<br>NHS-Blood and Transplant  |                  |
| 10.00-11.00<br>Sala Marivent |  | gene therapy to market AUDE in Ruiz, Abeona Therapeutics, Inc., Cleveland, OH   | NTES <b>&gt;</b> |
|                              | INV027   | Advancing AAV gene therapy for neuromuscular disea<br>example of X-linked myotubular myopathy<br>Fulvio Mavilio, Audentes Therapeutics Inc, San Francisc  |                  |
|                              | INV028   | Gene therapy for lisosomal storage diseases<br>Juan Ruiz, Abeona Therapeutics, Inc., Cleveland, OH  |                  |
| 11.00-11.30                  | Coffee bi  | reak  |                  |
|                              | Parallel s   | essions   |                  |
| 11.30-13.00<br>Sala Vent     |  | a - Cell and gene therapy of metabolic diseases<br>ima Bosch, Autonomous University of Barcelona  | VIRALGEN         |
|                              | INV029   | Gene therapy for hepatic metabolic inherited disorde<br>AAV vectors, Wilson disease<br>Gloria González-Aseguinolaza, CIMA, Pamplona   | rs using         |
|                              | INV030   | Pancreatic progenitors in the adult pancreas<br>Meritxell Rovira, <i>CMRB</i> , <i>Barcelona</i>  |                  |
|                              | OR013  | Counteraction of obesity and insulin resistance by live AAV-mediated BMP7 overexpression Veronica Jimenez, Universitat Autònoma de Barcelona; de Investigación Biomédica en Red de Diabetes y Enferm Metabólicas Asociadas (CIBERDEM) | Centro           |
|                              | OR014  | Curing disease before birth: in utero gene therapy for treatment of hereditary tyrosinaemia type 1 Clara Nicolas, Mayo Clinic, Rochester, MN  | the              |
|                              | OR015  | Gene-cell therapy for Pompe disease<br>Juan Elías González-Correa, <i>GENyO</i> , <i>Granada</i>  |                  |

| 11.30-13.00              | Parallel 3                                  | b - Cell and gene therapy of cardiovascular and bone diseases  |
|--------------------------|---|--|
| Sala Mar                 | Chair: Pilar Sepúlveda, IIS-La Fe, Valencia |  |
|                          |   | EuroClone®: Company Presentation   |
|                          | INV031                                      | Bmi1+ adult cardiac progenitor cells are critical for <i>in vivo</i> heart function after acute myocardial infarct Antonio Bernad, <i>CNB-CSIC</i> , <i>Madrid</i>   |
|                          | INV032                                      | Therapeutic potential of stem cells derived exosomes in cardiovascular diseases Pilar Sepúlveda, <i>Instituto Sanitario La Fe, Valencia</i>  |
|                          | OR016                                       | Directed mutagenesis of SUMOylation sites in N1ICD affects to cellular behavior: implications in cell therapy strategies Maria Ciria, Instituto de Investigación sanitaria La Fe/Centro de Investigación Príncipe Felipe, Valencia |
|                          | OR017                                       | Exosomes function as free fatty acid transporter<br>Akaitz Dorronsoro, Mixt Unit for Cardiovascular repair IIS La Fe-<br>CIPF, Valencia  |
|                          | OR018                                       | Gene induced HOXB7 in marrow mesenchymal progenitors improves performance by autocrine and paracrine mechanisms llenia Mastrolia, <i>Università di Modena e Reggio Emilia</i>  |
| 13.00-14.30              | Lunch                                       |  |
| 14.30-16.00<br>Sala Vent |   | a - Cell and gene therapy of blood disorders<br>in A. Bueren, CIEMAT/CIBERER/IIS-FJD, Madrid   |
|                          | INV033                                      | Gene therapy for pyruvate kinase deficiency<br>José Carlos Segovia, CIEMAT/CIBERER/IIS-FJD, Madrid   |
|                          | INV034                                      | Therapeutic strategies based on cell therapy within the haematopoietic stem cell transplant setting José Antonio Perez Simón, <i>Junta de Andalucía, Seville</i>   |
|                          | OR019                                       | Gene therapy for recombinase deficient-SCID<br>Laura Garcia Perez, <i>Leiden University Medical Center</i>   |
|                          | OR020                                       | Gene editing for pyruvate kinase deficiency: improvements towards its clinical application Sara Fañanás Baquero, CIEMAT; CIBERER; IIS-FJD/UAM, Madrid  |
|                          | OR021                                       | Development and potential applications of a haematopoietic stem cell model of X-linked dyskeratosis congenita Carlos Carrascoso-Rubio, CIEMAT; CIBERER; IIS-FJD/UAM, Madrid  |

# **PROGRAMME**

| 14.30-16.00              |           | b - Cell and gene therapy of skin and muscle  | Euro Clone serving science through innovation  |
|--------------------------|-----------|---|--|
| Sala Mar                 |           | der Izeta, Biodonostia, San Sebastián-Donostia  | and the state of t |
|                          | INV035    | Understanding muscle stem cell regenerative decline ageing Pura Muñoz-Cánoves, UPF-ICREA Barcelona/CNIC M   |  |
|                          | INV036    | Gene therapy of skin diseases<br>Fernando Larcher, CIEMAT/CIBERER/IIS-FJD, Madrid   |  |
|                          | OR022     | Age-associated dermal stem cell niche dysfunction c<br>with reduced Sox2 levels<br>Laura Yndriago, I.I.S. Biodonostia, San Sebastián-Donos  |  |
|                          | OR023     | CRISPR/Cas9-based gene editing approaches for the correction of recessive dystrophic epidermolysis bull derived-epidermal stem cells Jose Bonafont, Carlos III University (UC3M), Madrid  |  |
|                          | OR024     | Specific expression of GDNF in muscles as gene ther for ALS<br>Belén García-Lareu, Institut de Neurociencies (INc) UAL<br>CIBERNED, Barcelona   |  |
| 16.00-16.30              | Coffee br | reak  |  |
| 16.30-18.00<br>Sala Vent | diseases  | a - Cell and gene therapy of neurodegenerative<br>an José Toledo Aral, IBiS - Instituto de Biomedicina de   | Abeona   |
|                          | INV037    | Antiparkinsonian cell therapy using carotid body<br>Juan José Toledo-Aral, <i>IBiS - Instituto de Biomedicina</i>   | de Sevilla   |
|                          | INV038    | 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1 1   |  |
|                          |           | AAV-mediated CYP46A1 gene therapy for Huntington Nathalie Cartier, INSERM, Paris  | on's disease   |
|                          | OR025     | 0 1,  | long-term  |
|                          | OR025     | Nathalie Cartier, INSERM, Paris  Protection against cognitive decline during aging by expression in the CNS of secreted-klotho Miguel Chillon, CREA; Vall d'Hebron Research Institute   | long-term<br>(VHIR);<br>ome mouse  |
|                          |           | Nathalie Cartier, INSERM, Paris  Protection against cognitive decline during aging by expression in the CNS of secreted-klotho Miguel Chillon, CREA; Vall d'Hebron Research Institute Institut de Neurocièncias (UAB), Barcelona  Rescuing retinal degeneration in Bardet-Biedl syndromodel | long-term (VHIR); ome mouse th, London inistration ow up   |

| 16.30-18.00<br>Sala Mar |   | b - Cell and gene immunotherapy niel Bachiller, CSIC, Mallorca  | es |
|-------------------------|---|---|----|
|                         | INV039  | Treating AIDS with TALENs Daniel Bachiller, CSIC, Mallorca  |    |
|                         | INV040  | CAR-T immunotherapy; from our experience to the future<br>Manel Juan, <i>Hospital Clínic de Barcelona</i> , <i>IDIBAPS</i>  |    |
|                         | OR028   | Enhanced anti-inflammatory potential of mesenchymal stromal cells mediated by the ectopic transient expression of CXCR4 an IL10 María Rosario Hervás, CIEMAT/CIBERER/IISFJD, Madrid   |    |
|                         | OR029   | Preclinical optimisation of CAR CD123-expressing T-cells for acute myeloid leukaemia Matteo Libero Baroni, <i>Josep Carreras Leukemia Research Institute</i> , Barcelona  |    |
|                         | OR030   | NK cells from different sources as a promising alternative for CAR-based immunotherapy against hematological cancers Lara Herrera, Cell Therapy and Stem Cells Group, Basque Center for Blood Transfusion and Human Tissues, Galdakao, Bilbao | r  |
| 18.00-19.30             | Poster session 2  |   |    |
| 19.30-20.00             | SETGYC general assembly   |   |    |
| 21.00                   | Gala dinner at Restaurante Caballito de Mar<br>Address: Paseo Sagrera 5, Palma de Mallorca<br>Coaches will be leaving from the Barceló Illetas Albatros hotel at 8.30pm |   |    |

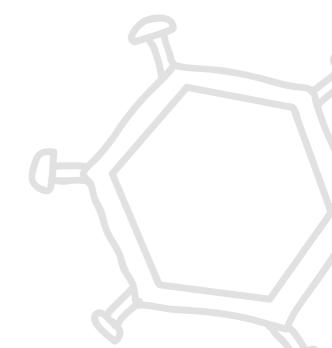
# **PROGRAMME**

# FRIDAY 16 MARCH 2018

|                         | Parallel so | ession   |
|-------------------------|-------------|--|
| 9.00-10.30<br>Sala Vent |             | a - Cell and gene therapy of cancer pen Hernandez, CIMA, Pamplona  |
|                         | INV041      | Oncolytic adenoviruses<br>Ramon Alemany, Catalan Institute of Oncology, Barcelona  |
|                         | INV042      | TIL therapy of cancer<br>Alena Gros, Vall Hebron Institute of Oncology, Barcelona  |
|                         | OR031       | Placental MSCs and their derivatives as vehicles for the Na/I<br>symporter (hNIS): a new antitumoural therapy<br>Maria del Pilar Martin-Duque, Instituto Aragonés de CC de la<br>Salud/ Fundación Araid, Zaragoza      |
|                         | OR032       | VCN-01 is a selective oncolytic adenovirus with hyaluronidase activity in patients with advanced or metastatic pancreatic cancer: first-in-human dose-escalation studies Manel Cascallo, VCN Biosciences SL, Barcelona |
|                         | OR033       | Remissions of spontaneous canine tumours after systemic<br>cellular viroimmunotherapy<br>Javier García-Castro, Unidad de Biotecnología Celular, Instituto de<br>Salud Carlos III, Madrid                               |
| 9.00-10.30<br>Sala Mar  |             | b - Rare diseases: leading gene and cell therapy<br>ria González Aseguinolaza, CIMA, Pamplona  |
|                         | INV043      | Systemic messenger RNA therapy as a treatment for acute intermittent porphyria Antonio Fontanellas, CIMA-University of Navarra, Pamplona   |
|                         | INV044      | Cell therapy in motor neuron diseases<br>Salvador Martínez, Institute of Neuroscience, Alicante  |
|                         | OR034       | Preclinical safety and efficacy evaluation of lentiviral transduced haematopoietic stem cells for the treatment of leukocyte adhesion deficiency type I Cristina Mesa-Núñez, CIEMAT; CIBERER; IIS-FJD/UAM, Madrid      |
|                         | OR035       | Phase I/II clinical trial for recessive dystrophic epidermolysis bullosa using EB-101 (COL7A1 gene-corrected autologous keratinocytes)  Jean Y. Tang, Stanford University, Stanford, CA                                |
|                         | OR036       | Preclinical studies towards the gene therapy of Diamond-Blackfan anaemia patients<br>Yari Gimenez-Martinez, CIEMAT/CIBERER/IISFJD, Madrid  |
| 10.30-11.00             | Coffee br   | reak   |

#### FRIDAY 16 MARCH 2018

|                              | Plenary session  |  |
|------------------------------|--|--|
| 11.00-12.30<br>Sala Marivent | <i>'</i>   |  |
|                              | INV045 Exploiting the therapeutic potential of haematopoietic stem cells gene editing for the treatment of inherited diseases Pietro Genovese, San Raffaele Telethon Institute for Gene Therapy, Milan |  |
|                              | INV046 Exosomes and mitochondria during intercellular communication through immune synaptic contacts Francisco Sánchez-Madrid, CNIC e Instituto de Investigación Sanitaria La Princesa, Madrid         |  |
| 12.30-13.45<br>Sala Marivent | 1  |  |
|                              | INVO47 A long and winding road to make from cells a surgical tool Damián García-Olmo, Hospital Universitario Fundación Jiménez Díaz, Madrid  |  |



# POSTER SESSION 1

# WEDNESDAY 14 MARCH, 20.00-21.30

| Poster no. | Poster title   |
|------------|--|
| P001       | Cell-based gene therapy to treat HIV-1/AIDS  |
| P003       | Adoptive cellular immunotherapy using CD1a CART-cells for treatment of cortical pediatric T-cell acute lymphoblastic leukaemia                     |
| P005       | Optimising culture conditions for adoptive cell therapy with lymphocytes   |
| P007       | Tumour infiltrating lymphocytes in pediatric patients: Analysis, expansion under GMP conditions and initial clinical experience                    |
| P009       | IL-10 gene hydrofection in human colon segments "ex vivo"  |
| P011       | Local immunotherapy with XCL1 and Flt3L expressed by recombinant Semliki forest virus (SFV) to therapeutically foster tumour antigen cross-priming |
| P013       | PHA-DHA nanoconjugates as a new strategy to prevent ischaemia-reperfusion injury in acute myocardial infarction                                    |
| P015       | Ischaemic damages modifies intercellular communication mediated by exosomes in heart   |
| P017       | The ex vivo transduction of human haematopoietic stem cells induces the expression of NKG2D ligands  |
| P019       | Optimised transduction of human haematopoietic progenitors to be applicable to a pyruvate kinase deficiency gene therapy clinical trial            |
| P021       | Oncolytic adenovirus expressing tumour neoepitopes as a vaccine  |
| P023       | Polypurine reverse Hoogsteen hairpins for silencing expression of cancer target genes in human cells   |
| P025       | Immunotherapy in paediatric sarcomas: treatment directed to metastasis initiating cells  |
| P027       | Modelling the clinical experience of children treated with Celyvir oncolytic virotherapy with the help of the TH-MYCN mouse                        |
| P029       | Correction of visual and auditory function by AAV9-mediated gene therapy in a mouse model of mucopolysaccharidosis type IIIB                       |
| P031       | Curative treatment of hereditary tyrosinaemia type 1 in pigs through <i>in-vivo</i> liver-directed lentiviral gene therapy                         |
| P033       | Modulation of astrogliosis in pluripotent and multipotent stem cells with GSK-inhibitors as a cell therapy of spinal cord injury                   |

| ъ .        | B + 191   |
|------------|---|
| Poster no. | Poster title  |
| P035       | Retinal organoids as models of tissue differentiation and retinal diseases  |
| P037       | Cerebellar transduction of astrocytes as gene therapy strategy for megaloencephalic leukoencephalopathy with subcortical cysts (MLC)        |
| P039       | Preclinical assessment of a hepatocyte-directed gene editing approach based on viral vectors and polymeric nanoparticles                    |
| P041       | Gene-repair of point mutations at the endogenous locus using polypurine reverse Hoogsteen hairpins in mammalian cells                       |
| P043       | Matrix metalloproteinase-9 downregulation by shRNA delivered in lipid nanoparticles as a potential strategy against corneal vascularisation |
| P045       | Spatiotemporal control of transgene expression using irradiation with near-infrared light   |
| P047       | Glutamine/glutamate metabolic adaptation during direct reprogramming of human fibroblasts to hepatocytes                                    |
| P049       | Efficacy of the administrations of autologous mesenchymal stromal cells in patients with complete or incomplete spinal cord injury          |
| P051       | Cell reprogramming for disease modelling of primary hyperoxaluria type 1  |
| P053       | Successful repeated hepatic gene delivery in non-human primates achieved with AAV5 by use of immunoadsorption                               |
| P055       | Therapeutic efficacy of VTX-801, an optimised AAV vector for the treatment of Wilson's disease  |
| P057       | Development of consistent GMP growth factors and cytokines for ATMP manufacture   |

<sup>\*</sup> Please note: even numbered posters will be presented in poster session 2



# POSTER SESSION 2

# THURSDAY 15 MARCH, 18.00-19.30

| Poster no. | Poster title   |
|------------|--|
| P002       | Celyvir, a cellular viroimmunotherapy, shows antitumoral efficacy using immunocompetent murine models                                    |
| P004       | Development of a hypoxia inducible gene expression system in mammalian cells   |
| P006       | Intravenous infusion of adipose-derived mesenchymal stromal cells induce an early immune response in the lungs                           |
| P008       | The combination of cell and gene therapy as tool to design a new generation therapy based on MSC derived exosomes                        |
| P010       | Generation of "off-the shelf" $\alpha$ CD19-CAR T cells for the treatment of B cell malignancies   |
| P012       | Idiotype vaccines produced with a non-cytopathic alphavirus vector induce anti-<br>tumour responses in a murine model of B-cell lymphoma |
| P014       | Unraveling Notch implication in exosome mediated angiogenesis of MDA 231 for the development of new therapies                            |
| P016       | Setting a safe harbour loading platform at the CCR5 locus in human cells   |
| P018       | Impact of mesenchymal stromal cell co-transplantation in HSC engraftment: comparison between two administration routes                   |
| P020       | Immunomodulation and anti-tumour potential of oncolytic adenovirus-loaded menstrual blood-derived mesenchymal stem cells                 |
| P022       | Arming oncolytic adenovirus with FAP-targeting bispecific T-cell engager to improve antitumour efficacy                                  |
| P024       | miR-99b and miR-485 are enhancers of adenoviral oncolysis in pancreatic cancer   |
| P026       | Oncolytic adenovirus with hyaluronidase activity that evades anti-adenovirus neutralizing antibodies: VCN-11                             |
| P028       | Effectiveness of cell therapy using neonatal cells for the treatment of liver disease  |
| P030       | Development and characterisation of a humanised porcine model of phenylketonuria   |
| P032       | Hydrofection of exogenous hAAT-flag in human liver segments mediates an expression as efficient as the endogenous gene                   |
| P034       | Gene therapy for Dravet syndrome: a proof of concept   |

| Poster no. | Poster title  |
|------------|---|
| P036       | Interrogating genetic predisposition to Parkinson's disease in iPS cells through CRISPR/Cas9-based genome edition               |
| P038       | Isolation and characterisation of human cremaster muscle stem cells   |
| P040       | Controlled deletions on the PKLR gene to generate a human haematopoietic progenitor model of pyruvate kinase deficiency         |
| P042       | Gene correction assisted by CRISPR/Cas9 system of the AGXT locus in a set of primary hyperoxaluria type 1 patients              |
| P044       | New approaches to deliver novel episcopal lentivirus (lentisoma) at high frequencies  |
| P046       | iPSC-derived astrocytes contribute to non-cell autonomous neurodegeneration in Parkinson's disease                              |
| P048       | Influence of different culture conditions on the chromosomal stability and pluripotent phenotype of human pluripotent stem cell |
| P050       | Patient's perspective on safety and efficacy of the administration of mesenchymal cells in spinal cord injury                   |
| P052       | Gene therapy restores normal weight in obese Bardet-Biedl syndrome 1 (BBS1) mouse model   |
| P054       | Nonclinical safety evaluation of VTX-801, an AAV vector for treatment of Wilson's disease                                       |
| P056       | Exploration of the non-coding transcriptome for mesenchymal stem cells characterisation   |
| P058       | UPV: production of customised high quality viral vectors at a joint UAB-VHIR technological platform                             |

<sup>\*</sup> Please note: odd numbered posters will be presented in poster session 1



# The ESGCT Interactive Gene and Cell Therapy Community Heatmap



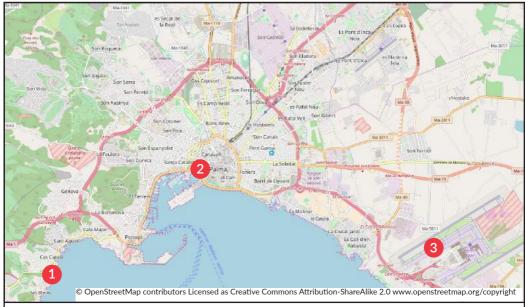
The ESGCT interactive Gene & Cell Therapy Community Heatmap shows where in Europe a growing number of academic institutions, biotech and pharmaceutical companies, non-profits and other organisations are working to bring new treatments to patients.

Learn who to ask for advice or find your next collaboration in a few clicks!

To view the heatmap and add your own organisation visit the ESGCT website

www.esgct.eu

#### MAP OF PALMA DE MALLORCA



1: Barceló Illetas Albatros hotel 2: Gala Dinner Venue 3: Airport

#### **SETGYC EVALUATION**

We do hope you have enjoyed the SETGYC Biennial Congress 2018. 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 by mid-April.

# Changing the Face of Modern Medicine: Stem Cells & Gene Therapy

16-19 October 2018 Swisstec EPFL, Lausanne

