CHANGING THE FACE OF MODERN MEDICINE: STEM CELLS & GENE THERAPY

PROGRAMME
ESGCT / ISSCR / SFTCG
COLLABORATIVE CONGRESS

16-19 OCTOBER 2018
SWISSTECH LAUSANNE

EUROPEAN SOCIETY OF GENE & CELL THERAPY

ISSCR INTERNATIONAL SOCIETY FOR STEM CELL RESEARCH

Société Française de Thérapie Cellulaire et Génique

XXVI ANNUAL CONGRESS | INTERNATIONAL 2018 SYMPOSIA | 15TH ANNUAL MEETING

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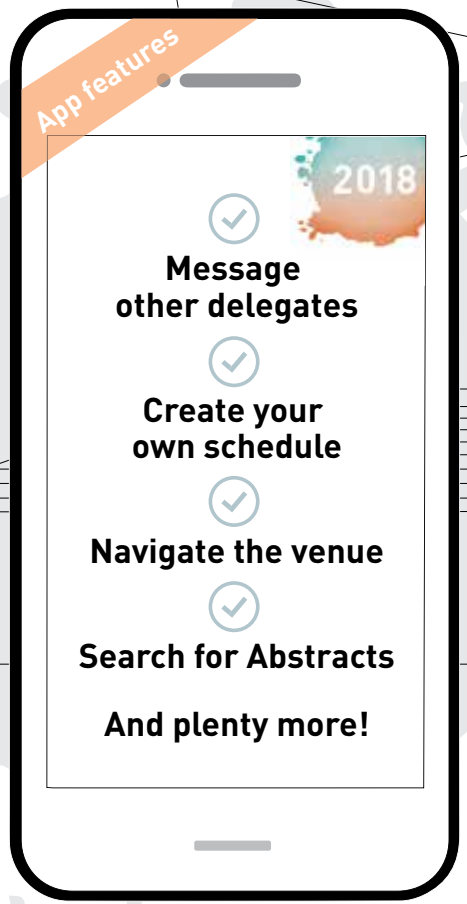
#CHANGINGMODERNMED
WHAT, WHO, WHEN & WHERE, AT YOUR FINGERTIPS...



HOW TO DOWNLOAD THE APP

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WELCOME

SWISSTECH CONVENTION CENTER

The SwissTech Convention Center is one of the largest conference centres in the Lake Geneva region hosting events of international fame. This majestic building is distinguished by its modernity and its innovative technologies. The architecture of the building allows three auditoriums to become one; the number of seats in each auditorium can also be changed in just a few minutes. This modularity is based on two mechanisms: a system of sliding walls, and the Gala Venue technology. The west facade of the building is covered with panels made of organic dye-sensitized solar cell, also called "Grätzel cells" after Michael Grätzel, a physical chemistry professor at the EPFL and the inventor of this technology.

REGISTRATION

Registration is located on the Campus floor, near the main entrance to the SwissTech Convention Center.

We will be available:

Tues 16th:	08.00 - 20.30
Weds 17th:	08.00 - 19.00
Thurs 18th:	08.30 - 19.30
Fri 19th:	08.30 - 19.30

WI-FI ACCESS

Wi-Fi is available throughout the SwissTech Convention Center.
Login: **Lausanne2018**
Password: **lausanne2018**

SPEAKER READY ROOM

The speaker ready room is Office A/B on the Garden Floor. When facing the cloakroom, the Speaker Ready Room is to your right.

USEFUL CONTACTS

Ambulance 144

Police 117

Fire brigade 118

Taxi: . . . . . +41 844 814 814

INFORMATION BOARD

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

ABSTRACTS AND DELEGATE LISTS

Electronic copies can be accessed through our congress app (please see inside front cover) or in **My Congress Materials** in your account on the ESGCT website.

Gaëlle Jamar, ESGCT manager office@esgct.eu
+44 776 647 5379

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WITH SPECIAL THANKS TO:

mco international Group

SwissTech Convention Center

FNSNF Fonds national suisse de la recherche scientifique

Lausanne Tourisme

JSGT

EPFL École polytechnique fédérale de Lausanne

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American Society of Gene & Cell Therapy

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www.7thfloor.co.uk

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WELCOME TO THE XXVITH ESGCT CONGRESS, LAUSANNE 2018

For the first time, the ESGCT Congress is being held in Switzerland. This year it is jointly organised with the International Society for Stem Cell Research (ISSCR), the French Society of Gene and Cell Therapy (SFTCG), and the Swiss local organising committee.

EUROPEAN SOCIETY OF GENE & CELL THERAPY

ISSCR INTERNATIONAL SOCIETY FOR STEM CELL RESEARCH

Société Française de Thérapie Cellulaire et Génique

The boards of ESGCT, ISSCR and SFTCG, along with the Swiss gene and cell therapy community, welcome you to Lausanne for the 2018 Annual Congress of ESGCT. The meeting will take place at the SwissTech Convention Center in the centre of the campus of the Ecole Polytechnique Federale de Lausanne (EPFL). The Center, characterised by its astonishing architecture and innovative technologies, will provide a superb environment for networking and sharing of exciting advances.

The scientific programme will highlight recent progress on ground-breaking technologies, outstanding pre-clinical proof-of-concept studies in gene and cell therapy, and updates on on-going clinical trials involving the wide range of conditions that are now targets for advanced therapies. To support exchange of information and expertise between various stakeholders and ensure dissemination of knowledge and expertise, we have organised a clinical trial & commercialisation workshop, along with educational sessions and public events. The meeting will also provide a unique opportunity to acknowledge the outstanding contributions of the members of the ESGCT and our partnering societies for their pioneering achievements in the fields of gene and cellular therapy.

We very much hope that you will attend the social events during the meeting, including the welcome reception, Molecular Mingle networking evening and closing drinks. This year the Molecular Mingle will be held at the Olympic Museum in the centre of Lausanne. The city has been the headquarters of the International Olympic Committee for more than a century, and the Olympic Museum has many fascinating exhibitions on sport and the history of the games. The museum will be for the sole use of our congress so we can enjoy each other's company whilst browsing.

During this meeting you will be in the heart of the Alps and on the shore of Lake Geneva. The region has an exceptionally strong economic, cultural, sporting and academic momentum. The stunning landscape of the eleventh century terraced vineyards of the Lavaux region, with the medieval village of St-Saphorin, has been recognised by UNESCO as a World Heritage Centre. The amazing Chillon castle, the most visited castle in Switzerland, is very nearby. The region is also rich in gastronomic traditions and has many musical and artistic attractions, including Chaplin's World with its magical journey through silent cinema, and the Montreux Jazz Festival.

As we look forward to the XXVIth ESGCT Congress and welcoming delegates from all over the world to Lausanne, we hope that you enjoy the meeting and thank you for your being part of it.

Robin R Ali
President, ESGCT

Doug Melton
President, ISSCR

Pierre Cordelier
President, SFTCG

Nicole Deglon
President, LOC

THANK YOU TO ALL OUR PARTNERS

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AWARDS

ESGCT OUTSTANDING ACHIEVEMENT

INV029: Didier Trono (2018 Outstanding Achievement Award lecture)
EPFL, Lausanne
Retroelements, their polydactyl controllers and the specificity of human biology

Human Gene Therapy
Mary Ann Liebert, Inc. publishers

ESGCT Young Investigator
OR031: Alessio Cantore (2018 Young Investigator Award lecture)
SR-TIGET, Milan
Shielding lentiviral vectors from phagocytosis increases hepatocyte gene transfer in non-human primates

AUDENTES

TRAVEL GRANTS

EUROPEAN SOCIETY OF GENE & CELL THERAPY

P060: Thomas Hartjes
Dept. of Neurosurgery, Erasmus MC, Rotterdam
Organotypic multicellular spheres (OMS) as a 3D model system to study oncolytic adenovirus responses in glioblastoma tumours

P023: Katelyn Masiuk
Department of Microbiology, Immunology and Molecular Genetics, University of California, Los Angeles, CA
dmPGE2 and poloxamer-F108 enhance transduction of human hematopoietic stem and progenitor cells with a ß-globin lentiviral vector

P357: Sem Aronson
UMC University of Amsterdam
AAV-mediated liver directed gene therapy corrects the cholestatic phenotype in Abcb4$^{-/-}$ mice

P298: Alessia De Caneva
ICGEB, Trieste
Coupling AAV-mediated promoterless gene targeting to SaCas9 nuclease to efficiently correct liver metabolic diseases

P396: Gabriele Ordazzo
San Raffaele Scientific Institute, Milan
Novel gene therapy approaches for whole brain delivery of the lysosomal GCase enzyme for wide protection from alpha-synuclein toxic aggregates

OR019: Anais Amaya
The University of Sydney, NSW
Successful *in vivo* editing of patient-derived primary human hepatocytes

P254: Valentina Vavassori
SR-TIGET, Milan
Optimization of a CRISPR/Cas9-based strategy for the correction of CD40LG gene in human haematopoietic stem cells and T cells

P255: Manel Llado
TIGEM, Naples
Homology-independent targeted integration for gene correction in photoreceptors

British Society for Gene & Cell Therapy

OR003: Micaela Harrasser
University College London
Effective targeting of ROR1+ solid tumours with next-generation Chimeric Antigen Receptor therapy

P483: Weiheng Su
University of Oxford
Exploiting adenovirus mechanisms for the enhanced production of AAV vectors

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

OR007: Kleopatra Rapti
Heidelberg University Hospital
Generation of novel immune-evading AAVs through identification and mutation of immunogenic epitopes in the variable capsid regions of adeno-associated Virus 9

P328: Razieh Monjezi
University Hospital Würzburg
CRISPR/Cas9 unites with Sleeping Beauty to generate CAR-T cells with enhanced therapeutic index for fighting against immunosuppressive tumour microenvironment

Sociedad Española Terapia Génica y Celular

P052: Carlos Carrascoso
CIEMAT/CIBERER-ISCIII, Madrid
Towards the gene therapy of the bone marrow failure in patients with dyskeratosis congenita

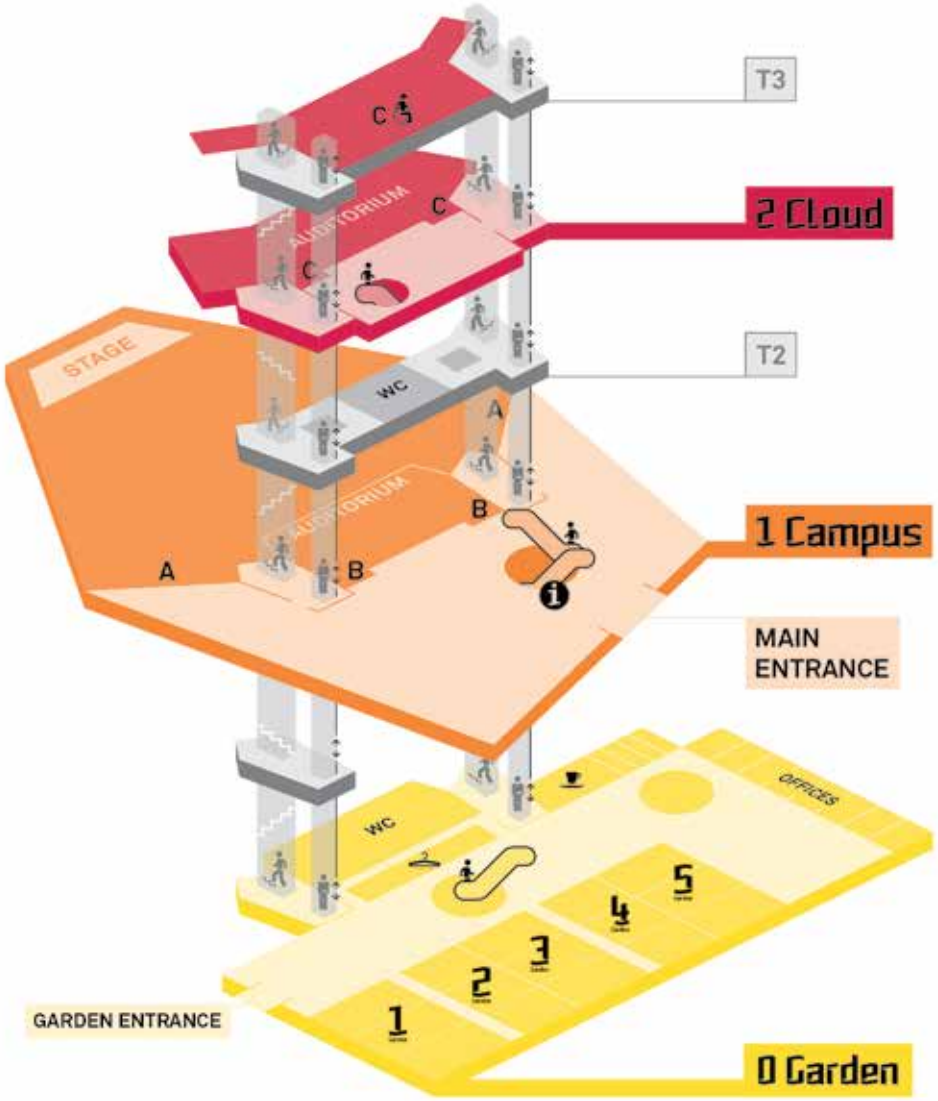
P027: Yari Gimenez Martinez
CIEMAT/CIBERER-ISCIII, Madrid
Preclinical studies towards the gene therapy of Diamond-Black-fan anemia

Société Française de Thérapie Cellulaire et Génique

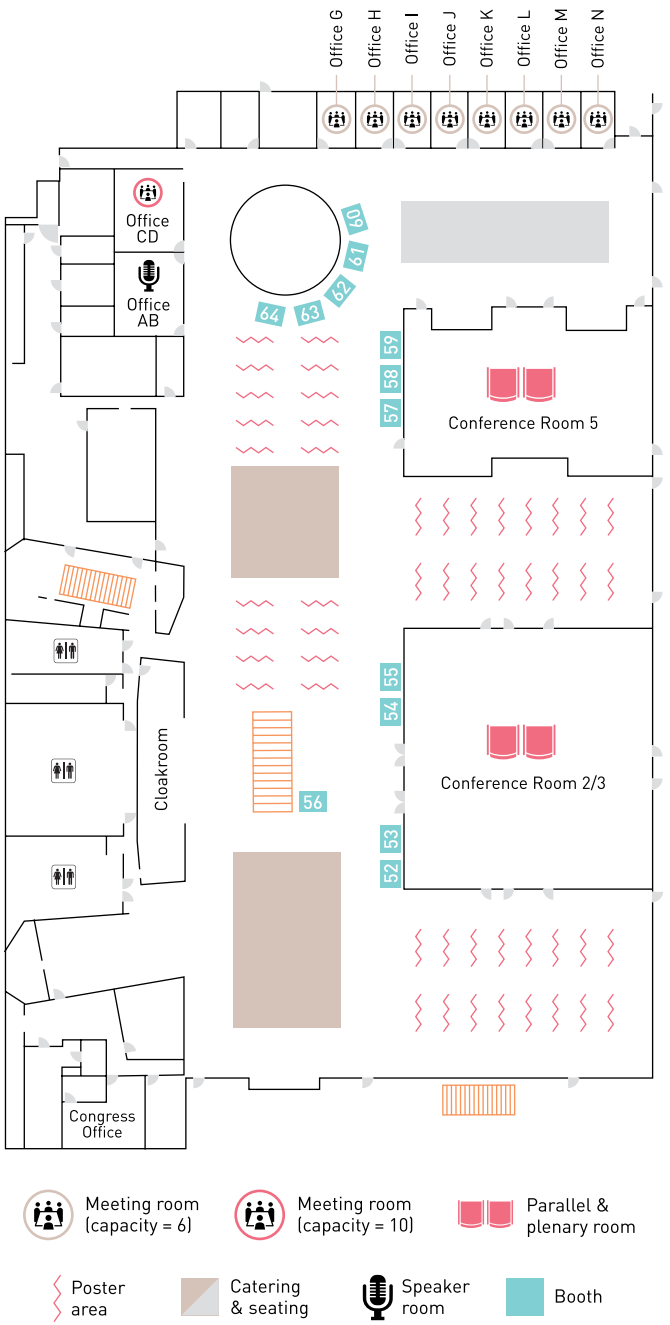
P109: Marine Charrier
INRA/Oniris UMR 703, Nantes
Demonstration of immunomodulatory properties for human MuStem cell population, a promising candidate for cell therapy of muscular dystrophies

P435: Sarah Le Saux
Institut Charles Gerhardt Montpellier, UMR 5253 CNRS-UM-ENSCM
Exploring the potential of extracellular vesicles as drug delivery systems

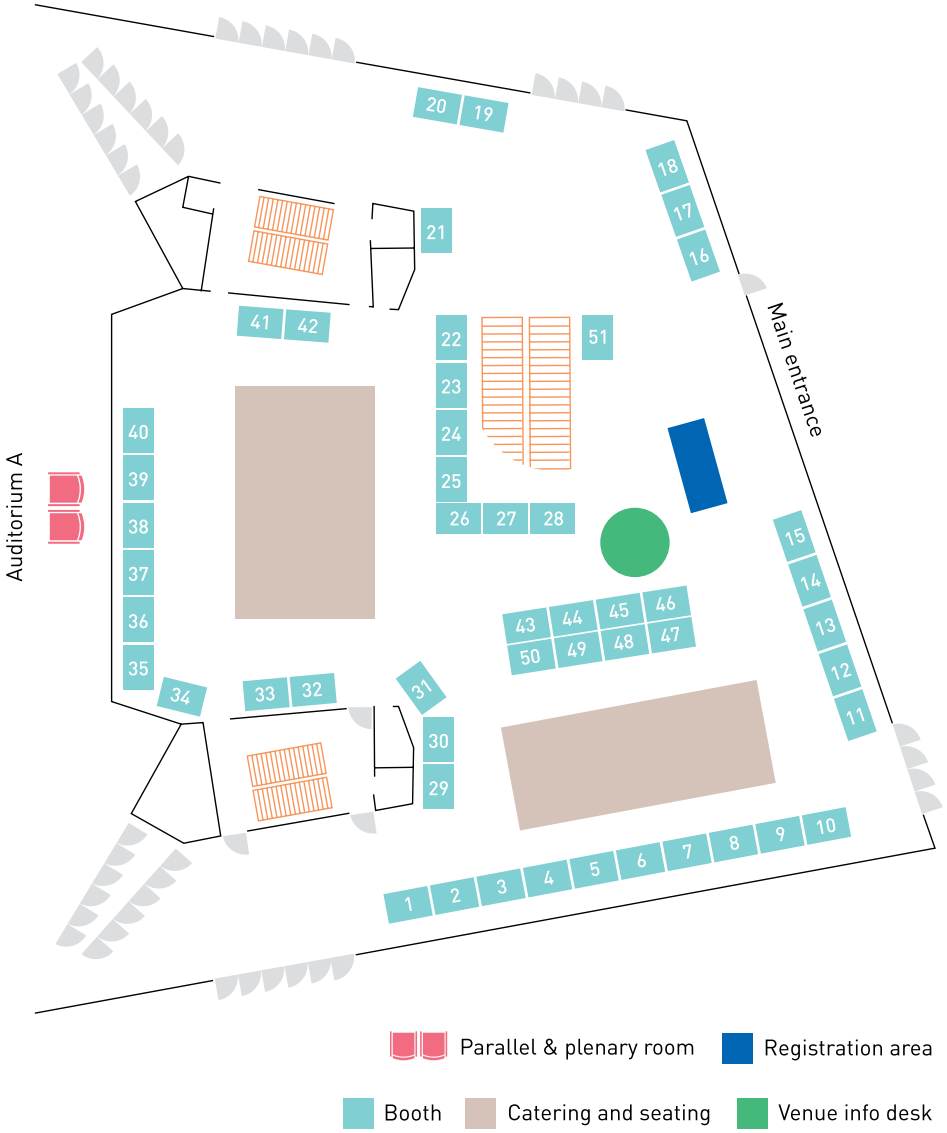
SWISSTECH : 3D FLOORPLAN



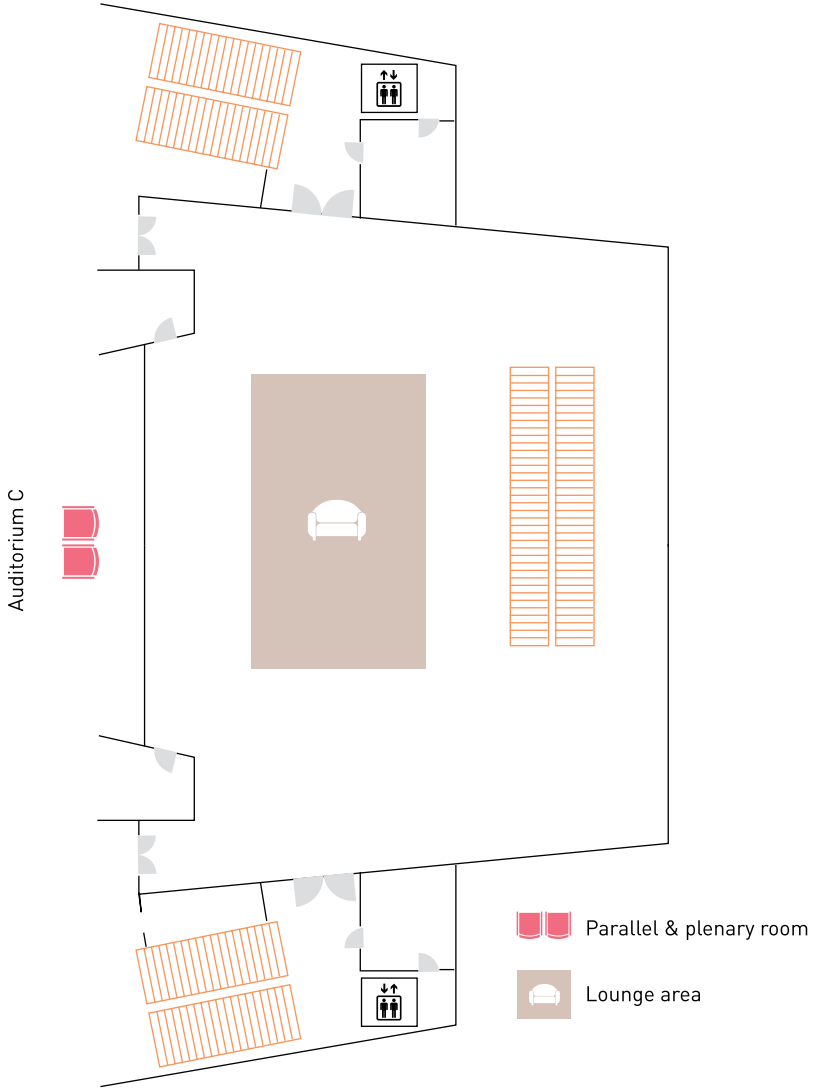
SWISSTECH : GARDEN FLOOR



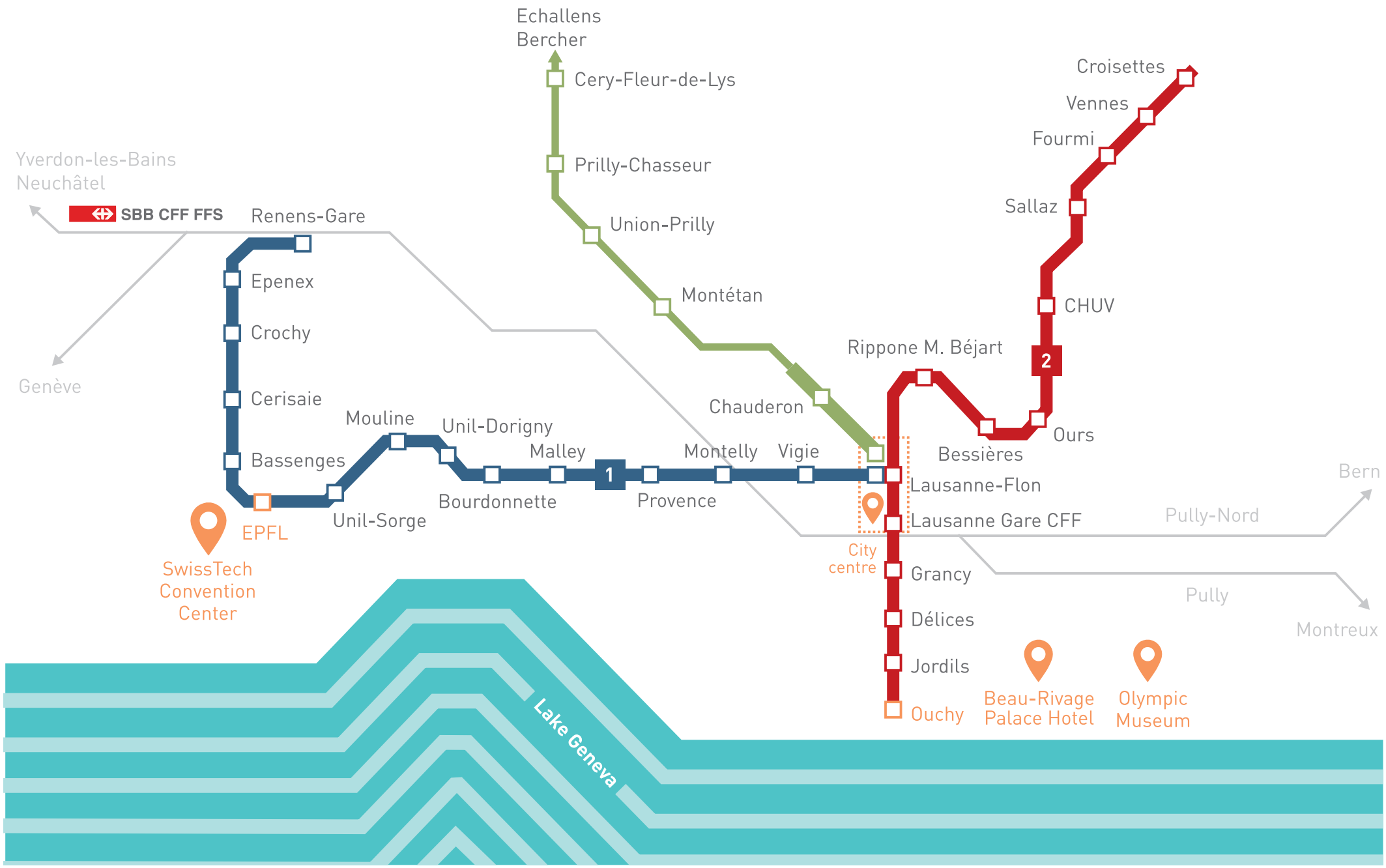
SWISSTECH : CAMPUS FLOOR

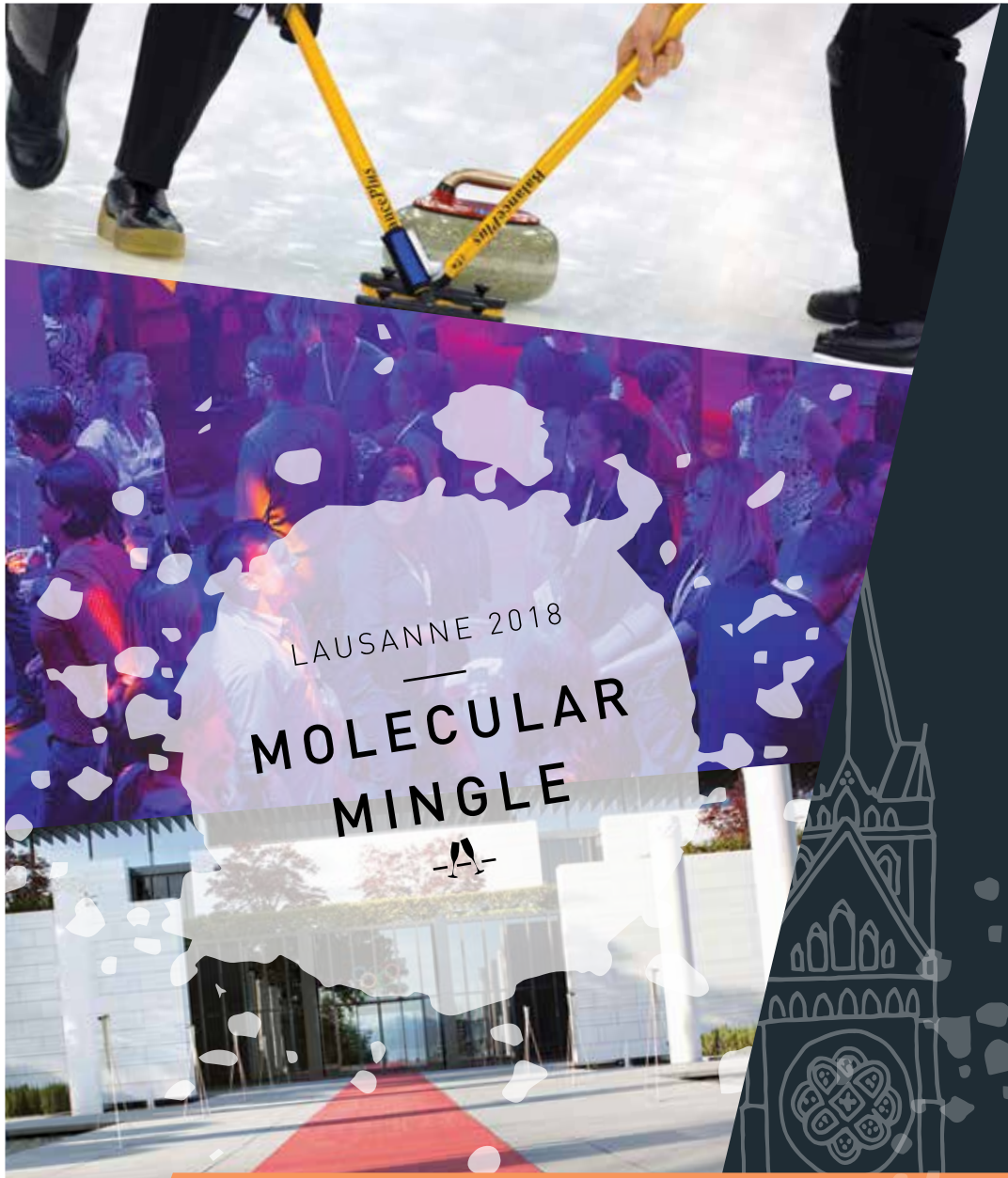


SWISSTECH : CLOUD FLOOR



CONGRESS LOCATIONS ON THE METRO





DON'T MISS THE ESSENTIAL NETWORKING HIGHLIGHT

THURSDAY 18 OCTOBER AT 8PM

OLYMPIC MUSEUM, LAUSANNE

curling /ˈkəːlɪŋ/

noun: a game played on ice in which large round flat stones are slid across the surface towards a target. Members of a team use brooms to sweep the surface of the ice in the path of the stone to control its speed and direction.

POLITE NOTICE: We WILL take pictures....and targets are NOT to be human!

Photobooth fun!

LAUSANNE 2018

MOLECULAR MINGLE

MEIRAGTx

Intellia THERAPEUTICS

Curling competition @ 9pm

Live music & disco

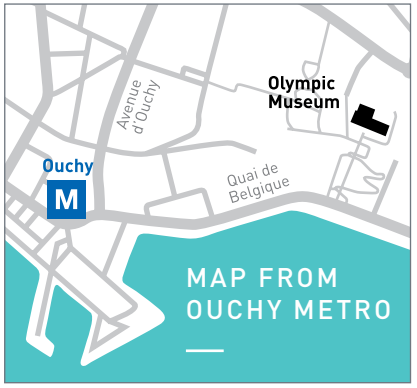
Photobooth

Food

Open bar until 23:00
Paying bar 23:00 - 02:00

Coaches will leave from SwissTech at 19:30 to the Olympic Museum

Easily accessible by public transport, don't forget to use your free travel pass! Make sure you join us at our (in) famous Molecular Mingle to network and socialise with friends old and new; and try your hand at curling...



EUROPEAN SOCIETY OF
GENE & CELL THERAPY

PROUDLY SUPPORTING THE TOWNSHIP PROJECT IN SOUTH AFRICA

ESGCT IS NOW WORKING with a selection of handpicked suppliers to ensure we are sourcing products which are environmentally friendly, support fair trade and offer sustainability and community inclusion.

Here at Lausanne 2018, we are proud to be distributing congress bags from the Township Project in South Africa. Township® was founded in 1997 with a commitment to creating meaningful and sustainable economic opportunities for women in South Africa's township communities.

Job creation and women's economic empowerment form the foundation of the Township® ethical fashion brand. All items are hand made, largely by women-run co-ops, which means that every purchase of a Township® product directly translates into work for township women. Materials are ethically sourced and Township® was the first South African fashion brand to become a member of the World Fair Trade Organization.

'The co-operatives are at the heart of everything we do at Township®. My aim was to bring economic opportunity to women in their own communities. Listening to them and trying to answer their needs has shaped my life radically. Working together, we have been on a journey of positive change.'
Township® founder & CEO,
Nicole-Marie Iresch

township®

MAKE YOUR WORDS BLOOM!

INSTEAD OF OUR USUAL ESGCT PENCIL, we have this year opted to bring you pencils you can - once used - plant and nurture into your very own ESGCT plant. With various options to choose from, which one did you get? Swap and trade with your colleagues, or come and see us at the ESGCT booth, where we also have some pencil sharpeners going if you need to sharpen up.

WHERE WE CAN, we work with suppliers who themselves work in a sustainable and community-centred way; our printed matter is made from green stock, our lanyards from bamboo. Where we can't, we aim to reuse and / or recycle as much as possible, so to this end remember to hand your badge wallets back into the registration desk – or drop them into the clearly marked boxes around the venue - before you head home after the congress!

IN LAUSANNE

ART BRUT MUSEUM

The concept of Art Brut stems from the French painter Jean Dubuffet who, from 1945, assembled a collection of objects created by the inmates of various psychiatric hospitals and prisons—solitary or outcast persons. In their creations, he saw "an entirely pure, raw artistic operation that the creator fully reinvents in all its phases, as spurred uniquely by his own impulses." The idea of Art Brut is thus based on certain social characteristics and aesthetic peculiarities. | **www.artbrut.ch**

HERMITAGE FOUNDATION

In 1841, the banker Charles-Juste Bugnion bought the land called The Hermitage, on a hill overlooking Lausanne. The superb view over the lake and the cathedral had already been immortalised by Camille Corot during his stay in 1825. The villa, with part of the park, was given to the City of Lausanne by the descendants in 1976. The mansion has been carefully restored to its original splendour.
A private foundation was born at the same time. Its twofold mission is:

- to keep a superb 19th-century Lausanne residence alive and open to the public
- to develop a high-quality museum of fine arts

| **www.fondation-hermitage.ch**

JUST OUTSIDE LAUSANNE

GRANDVAUX, CULLY & LES EPESSES

With their narrow streets and balconies over Lake Geneva, those 3 villages are 3 of the most typical villages in the Lavaux UNESCO region. Composed of several compact groups of winegrowers' houses, the villages have a characteristic but not typical silhouette. With narrow alleys and terraces overlooking the vines, the lake and the Alps, the experience is unforgettable. There are many walks between those villages one from Grandvaux to Cully for example, you can take a train from Lausanne to Grandvaux and could start with a lunch at the Auberge de la Gare.
| **www.aubergegrandvaux.ch/restaurant** |
More details on the walk can be found on the Lausanne Tourism website: www.lausanne.ch/en/thematiques/loisirs-et-de-tente/pour-les-lutins/balades-et-visites/balades-thematiques/vignoble-de-lavaux/de-grandvaux-a-cully.html

LUTRY

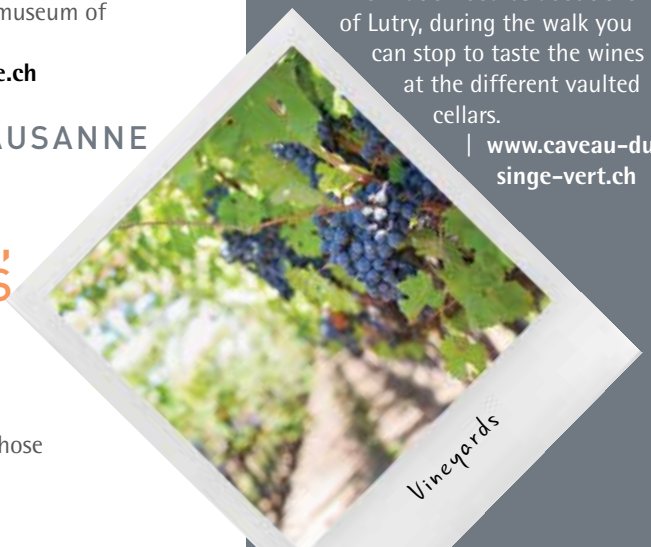
Even closer to Lausanne, just on the shores of the Lake this small medieval town is of great historical importance. The well-preserved heart of the town, with its narrow alleyways and numerous merchants' and noblemen's houses dating from the 15th to 18th centuries, is under a preservation order. There is a marked circular walk with eleven information boards about the history of Lutry, during the walk you can stop to taste the wines at the different vaulted cellars.
| **www.caveau-du-singe-vert.ch**

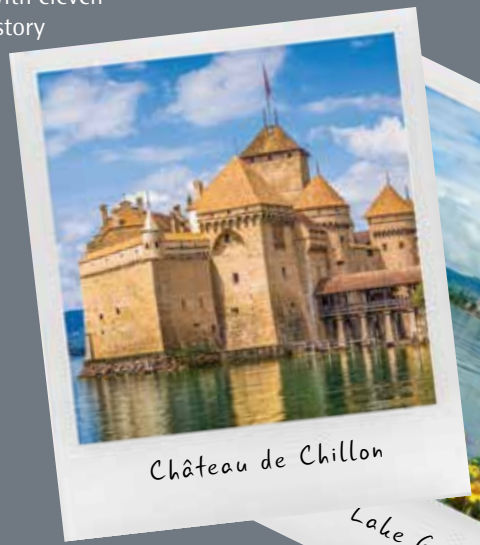
CHÂTEAU DE CHILLON

Further out, very near Montreux, it is worth visiting this Mediaval Castle. This Castle is the result of several centuries of constant building, adaptations, renovations and restorations, this site has been occupied since the Bronze Age. The rocky island on which the castle is built, was both a natural protection and a strategic location to control the passage between northern and southern Europe. The history of the castle was influenced by three major periods.
| **www.chillon.ch/en/GP765/history**

CHARLIE CHAPLIN MUSEUM

Meet Charlie Chaplin, family man, in the Manoir in Montreux; embark on a singular journey through the sets of his greatest films at our Hollywood-style Studio; take a peaceful walk in the gardens, get ready for an unforgettable experience!
| **www.chaplinsworld.com**





HASHTAG TO WIN!

TIME TO GET SOCIAL

Stay on top of everything that's happening at the congress by following us on Facebook, Twitter and Instagram! Make sure you use the official hashtag **#ChangingModernMed** to find and share the latest updates, get in touch or win a prize in our competitions.

Check out the ESGCT and ISSCR booths as well, here you can find out about upcoming events, join in competitions, play games, get more involved in YOUR society or just say hi! At the ESGCT booth you will also be able to buy tickets for the Molecular Mingle.

PRIZES

Win Molecular Mingle tickets, Giant Microbes or one of our other top secret prizes for:

- **The best Congress photo**
- **The best Lausanne photo**
- **The Molecular Mingle selfie with the most people**
- **The most retweeted Congress tweet**
- **The most liked Facebook post**

Only posts tagged **#ChangingModernMed** will be considered and keep your privacy settings in mind - if we can't see your posts, we can't include them in the competition!

ACTIVITIES

Drop by the ESGCT booth to join in the social media competitions, the vox populi poster prize for young investigators, or meet our student board members.

DID YOU KNOW?

That you can contact other delegates through our app (see page inside front cover) and get tweeting, FBing and 'gramming straight from the app?

THE SMALL PRINT

Please be courteous and considerate in your use of social media and always make sure that you don't share unpublished content without the author's consent.

ESGCT: www.facebook.com/ESGCT/
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ESGCT AWARDS

ESGCT outstanding achievement

ESGCT travel grants for best scoring abstract

ESGCT young investigator
(sponsored at national society meetings)

ESGCT young investigator

Vox populi: best poster

NEW AWARDS

Just launched – apply soon!
Please check the ESGCT website for full information and how to apply.

ESGCT mentor award
A mentor is often the most overlooked, yet most valuable person in ensuring a PhD student's progression and survival! This is the chance for individuals who have benefited from effective mentoring to give back. €500 cash award, and complimentary registration to annual congress. Applications open in April.

ESGCT public engagement award
Finding ways to share your research and to get people from outside the scientific community involved is an increasingly important part of the research process. ESGCT wants to help promote excellent work. €500 cash award, and complimentary registration to annual congress. Applications open now!

ESGCT travel grants have increased from ten €300 grants to twenty €500 grants

BLOG

The ESGCT blog is written by members and guest bloggers and covers all themes related to gene and cell therapy; from fundamental science to patient and public engagement. Read it on our website and get in touch if you would like to contribute!

FORUM

Newly launched, the ESGCT Forum is the place to keep in touch before, during and after congresses and spring schools, look and post opportunities for jobs and conferences but more importantly talk about gene and cell therapy. Discuss new papers, ask questions about techniques, and post your protocols!

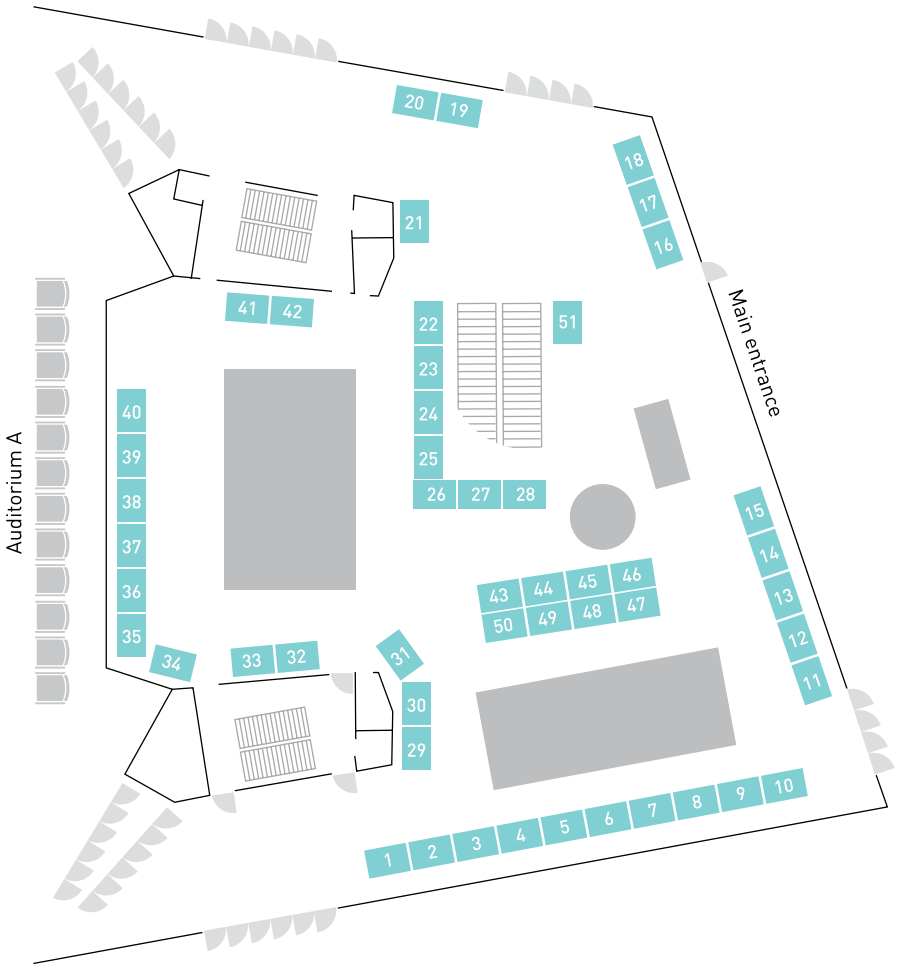
STUDENT BOARD MEMBERS

Our student board members Joost van Haasteren and Larisa Condurat represent early career researchers at ESGCT board level. If you have any comments and ideas, please send them their way. You can email them: joost@esgct.eu and larisa@esgct.eu or better yet, come see them at the ESGCT booth!

THE INTERACTIVE GENE & CELL THERAPY COMMUNITY HEATMAP

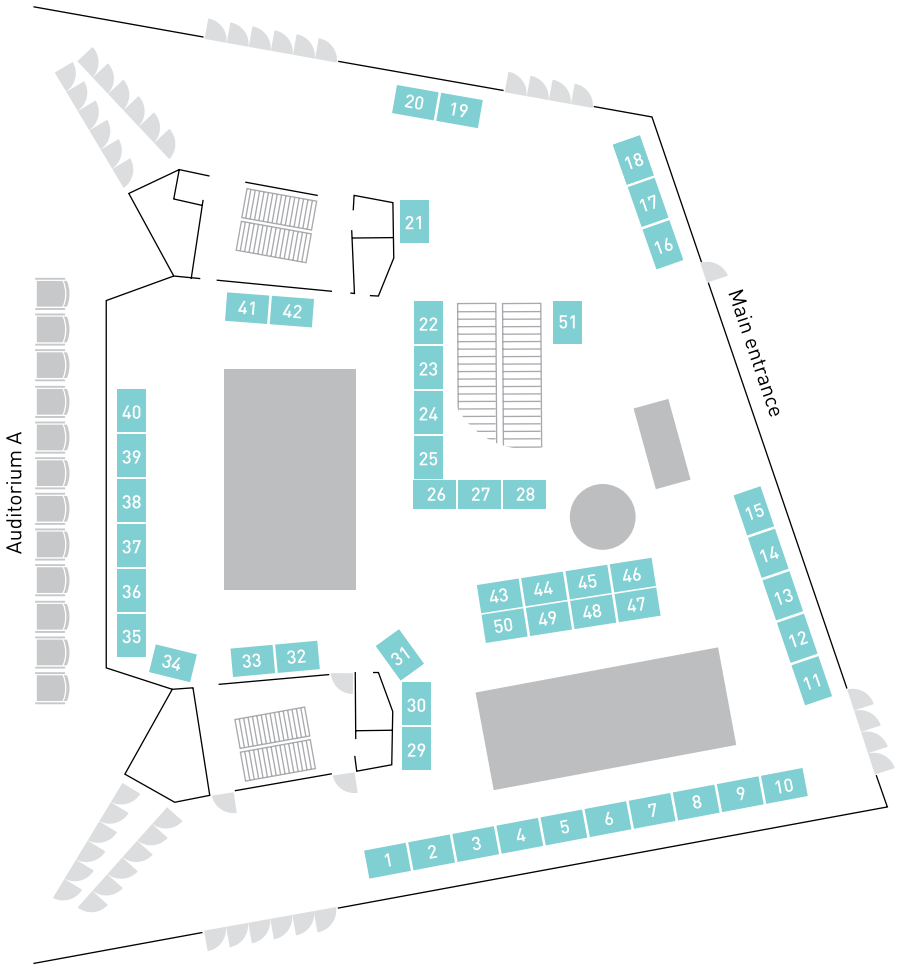
The interactive gene & cell therapy community heatmap on our website shows where in Europe a growing number of academic institutions, biotech and pharmaceutical companies, non-profits and other organisations are working to bring new treatments to patients. Learn who to ask for advice or find your next collaboration in a few clicks!

CAMPUS FLOOR EXHIBITORS 1-26



1	PlasmidFactory
2	SIRION BIOTECH
3	PROGEN passion for research
4	gene WERK
5	ArcticZymes
6	BI Biological Industries
7	EuroClone serving science through innovation
8	flash THERAPEUTICS
9	NHS Blood and Transplant
10	PEPROTECH OUR SUPPORT, YOUR DISCOVERY
11	TaKaRa Contech TaKaRa cellartis
12	cevec
13	AMERICAN SOCIETY of GENE & CELL THERAPY
14	STEMCELL TECHNOLOGIES
15	Gene Therapy Center Austria
16	MACS Miltenyi Biotec
17	biotechne
18	SYNTHACE
19	Penn Medicine GENE THERAPY PROGRAM
20	Vironova
21	SYNENTEC
22	VIVE biotech
23	chemometec
24	Caltag Medsystems life science solutions
25	brammer bio
26	OxfordBioMedica

CAMPUS FLOOR EXHIBITORS 27-51



27	BIONTECH Innovative Manufacturing Services
28	Polyplus transfection
29	PALL Biotech
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31	Human Gene Therapy Mary Ann Liebert, Inc. publishers
32	MOLMED
33	novasep
34	FinVector Vision - Therapies
35	KCT Kuopio Center for Gene and Cell Therapy
36	MDPI
37	GenoSafe
38	ALS Automated • Lab • Solutions
39	ASPHALION
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43	MaxCyte
44	DELPHI genes
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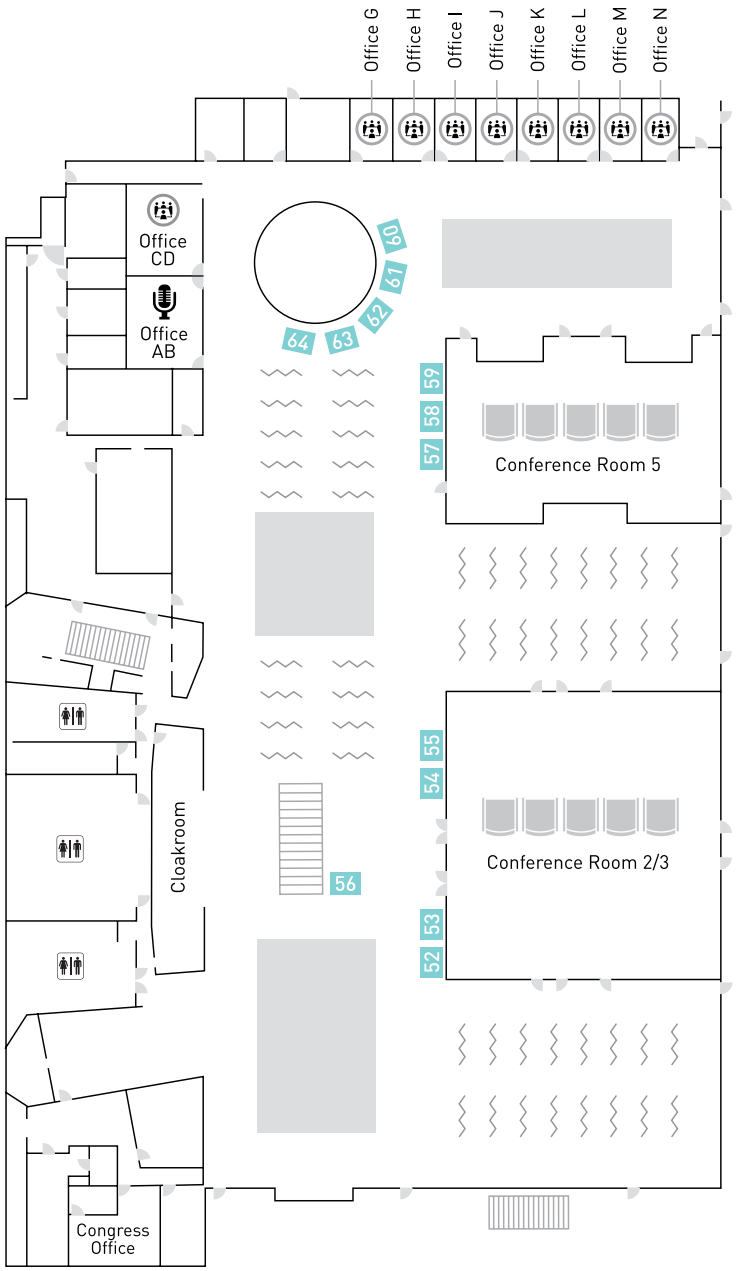
46	SAREPTA THERAPEUTICS
47	XENDO SOLUTIONS FOR LIFE SCIENCES
48	ASEPTIC TECHNOLOGIES
49	YPOSKESI
50	bluebirdbio
51	SYNTHEGO

GARDEN FLOOR EXHIBITORS 52-64



52	AJINOMOTO
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54	Orchard therapeutics
55	amsbio
56	GenIbet Biopharmaceuticals / Clean Cells
57	JENAL & PARTNERS BIOSAFETY CONSULTING
58	NATURE TECHNOLOGY Biologics by Design
59	ALFA WASSERMANN SEPARATION TECHNOLOGIES
60	ROweMed Medical 4 Life
61	OXFORD GENETICS BIOLOGY ENGINEERED
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British Society for Gene & Cell Therapy

The British Society for Gene and Cell Therapy (BSGCT) is dedicated to facilitating education, communication and the sharing of expertise and knowledge

Promoting Excellence | Education and Training
Nurturing Early Career Researchers
Public Engagement | Collaboration and Networking

Images courtesy of Simon Callaghan Photography

www.bsgct.org

Upcoming events

BSGCT Autumn Conference 2018

23 November 2018, London

Join us for this one-day conference as we explore genome editing for gene and cell therapy: technologies, models and translation

BSGCT Early Career Development and Collaboration Day & Joint ISCT Evening Networking Reception

co-hosted by the NIHR GOSH BRC Junior Faculty

22 November 2018, London

A daytime event for Early Career Researchers will be rounded off with a networking reception open to all. This is a FREE event, but places are limited!

UCL Great Ormond Street Institute of Child Health
NHS National Institute for Health Research Biomedical Research Centre-Junior Faculty
International Society ISCT Cell & Gene Therapy

BSGCT Annual Conference 2019

19 June - 21 June 2019, Sheffield

Highlighting the latest research and techniques in gene and cell therapy, including presentations from Stephan Grupp, Kathrin Meyer and Michele de Luca

Join us!

Become a BSGCT member to receive reduced rates for conferences and events, and much more. View our membership benefits at: www.bsgct.org

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/BSGCT

PARTNERS

DIAMOND

BioReliance | Merck
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 **www.bioreliance.com**

Oxford BioMedica (LSE:OXB) is a leading gene and cell therapy group focused on developing life changing treatments for serious diseases. Oxford BioMedica and its subsidiaries (the "Group") have built a sector leading lentiviral vector delivery platform (LentiVector®), which the Group leverages to develop *in vivo* and *ex vivo* products both in-house and with partners. The Group has created a valuable proprietary portfolio of gene and cell therapy product candidates in the areas of oncology, ophthalmology and CNS disorders. The Group has also entered into a number of partnerships, including with Novartis, Bioverativ, Sanofi, Axovant, Orchard Therapeutics, Boehringer Ingelheim/UK Cystic Fibrosis Gene Therapy Consortium/Imperial Innovations, GC LabCell and Immune Design, through which it has long-term economic interests in other potential gene and cell therapy products. Oxford BioMedica has world class facilities and capabilities to support pre-clinical, research and bioprocessing development through to GMP production and supply of commercial and clinical trial materials. The production activities are focused on the manufacture of lentiviral vectors from human cell lines in large scale serum free suspension cultures. Oxford BioMedica is based across several locations in Oxfordshire, UK and employs more than 320 people. Further information is available at **www.oxfordbiomedica.co.uk**

PARTNERS

PLATINUM

Adverum is a clinical-stage gene therapy company targeting unmet medical needs in serious rare and ocular diseases. Adverum has a robust pipeline that includes product candidates designed to treat rare diseases alpha-1 antitrypsin (A1AT) deficiency and hereditary angioedema (HAE), as well as wet age-related macular degeneration (wAMD). By leveraging our core capabilities, which include clinical development and in-house manufacturing expertise, we are moving product candidates into the clinic that are designed to provide durable efficacy with the potential to greatly improve the quality of life for patients. Adverum has collaboration agreements with Regeneron Pharmaceuticals to research, develop, and commercialize gene therapy products for ophthalmic diseases, and Editas Medicine to explore the delivery of genome editing medicines for the treatment of inherited retinal diseases. **www.adverumbio.com**

bluebird bio is a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic diseases and T cell-based immunotherapies. The company's objective is to develop and bring to market the most advanced products based on the transformative potential of gene therapy to provide patients hope for a better life in the face of limited or no long-term safe and effective treatment options. Headquartered in Cambridge, Massachusetts, bluebird bio has operations in the U.S. and Europe. **www.bluebirdbio.com**

The Human Induced Pluripotent Stem Cells Initiative (HipSci) is generating a large, high-quality reference panel of human iPSC lines for the research community. HipSci stands apart from other iPSC projects in a number of respects:

- **Magnitude** – HipSci is systematically generating iPSCs from hundreds of donors using a standardised experimental pipeline.
- **Cell line availability** – HipSci's cell lines are produced as a global iPSC resource. The cell lines are readily available from two cell banks (ECACC and EBiSC), for use by the wider research community.
- **Cohorts** – HipSci's reference panel comprises hundreds of cell lines from phenotypically healthy donors, plus several cohorts of donors with inherited genetic diseases.
- **The data** – Each line generated is extensively characterised. Assays include genotyping by array, expression array, methylation array, RNA-seq, Exome-seq, proteomics mass spect
www.hipsci.org

PARTNERS

PLATINUM

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

Lonza offers world-class technology platforms in the areas of GMP cell culture and viral-based therapeutic manufacturing, custom bio-therapeutic culture media, a large selection of primary and stem cells and a full line of custom bioassays. Our extensive experience in cell therapy process optimisation and scale-up innovation helps clients to safely and effectively advance their products through all phases of the commercial pipeline and maximise their return on investment. Our new viral-based therapeutics group provides viral vaccine manufacturing as well as viral vector mediated gene therapies. Our staff can design, develop and implement a manufacturing process that meets your autologous or allogeneic therapeutic applications.
www.pharma.lonza.com

MeiraGTx is a vertically integrated, clinical stage gene therapy company with four ongoing clinical programs and a broad pipeline of preclinical and research programs. MeiraGTx has core capabilities in viral vector design and optimization and gene therapy manufacturing, as well as a potentially transformative gene regulation technology. Led by an experienced management team, MeiraGTx has taken a portfolio approach by licensing, acquiring and developing technologies that give depth across both product candidates and indications. MeiraGTx's initial focus is on three distinct areas of unmet medical need: inherited retinal diseases, severe forms of xerostomia and neurodegenerative diseases. Though initially focusing on the eye, salivary gland and central nervous system, MeiraGTx intends to expand its focus in the future to develop additional gene therapy treatments for patients suffering from a range of serious diseases.
www.meiragtx.com

PARTNERS

PLATINUM

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas. REGENXBIO is currently developing five product candidates, including RGX-314 for the treatment of wet age-related macular degeneration (wet AMD), RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH), RGX-111 for the treatment of mucopolysaccharidosis type I (MPS I), RGX-121 for the treatment of mucopolysaccharidosis type II (MPS II) and RGX-181 for the treatment of late-infantile neuronal ceroid lipofuscinosis Type 2 (CLN2 Batten disease).
www.regenxbio.com

Rocket Pharma is a clinical-stage gene therapy company with a multi-platform approach for the treatment of patients with devastating diseases. We leverage LV 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 and manufacturing partnerships and 3) a laser focus on optimizing and innovating gene therapy product parameters through a seasoned scientific approach that de-risks programs as they enter clinical trials. **www.rocketpharma.com**

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates and is proud to support the 2018 ESGCT Annual Meeting. For more information about Sarepta. **www.sarepta.com**

At Spark Therapeutics, a fully integrated, commercial company committed to discovering, developing and delivering gene therapies, we challenge the inevitability of genetic diseases, including blindness, hemophilia and neurodegenerative diseases. We have successfully applied our technology in the first FDA-approved gene therapy in the U.S. for a genetic disease, and currently have three programs in clinical trials, including product candidates that have shown promising early results in patients with hemophilia. At Spark, we see the path to a world where no life is limited by genetic disease. For more information, visit **www.sparktx.com**, and follow us on Twitter and LinkedIn.

PARTNERS

GOLD

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

Brammer Bio is a best-in-class viral vector contract development and manufacturing organization (CDMO) for companies developing gene-modified cell therapies and *in vivo* gene therapies. Brammer offers end-to-end CDMO services from Phase I/II in Florida through commercial manufacturing in Massachusetts. Brammer Bio provides clinical and commercial supply of viral vectors for advanced therapies, process and analytical development, and regulatory support, enabling large pharma and biotech clients to accelerate the delivery of novel medicines to improve patient health. The Brammer team consists of extraordinary employees focused exclusively on serving the gene therapy needs of clients and their patients. We are Helping to Cure.®
www.brammerbio.com

Editas Medicine is a leading genome editing company focused on translating the power and potential of the CRISPR/Cas9 and CRISPR/Cpf1 genome editing systems into a robust pipeline of treatments for people living with serious diseases around the world. Editas Medicine aims to discover, develop, manufacture, and commercialize transformative, durable, precision genomic medicines for a broad class of diseases. For the latest information and scientific presentations, please visit
www.editasmedicine.com

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PARTNERS

GOLD

Available Gene therapy relevant development and manufacturing capabilities and capacities can hardly support the strong growth of gene therapy product development activities across the globe. Long process development times and low yield manufacturing processes slow down product development and add significant costs and risks to the development and licensure of gene therapy products.
The Gene Therapy Center Austria (GTCA) covers all steps from vector to GMP production (200L, 500L, and 1000L) until early stage commercial including a broad range of AAV specific analytical methods.

GTCA's one-stop-shop concept is based on Shire's proprietary AAV technology platform. The GTCA has successfully demonstrated lot-to-lot consistency and scalability across multiple programs.

Our partners benefit from our 30+ year experience in development, approval and global supply of complex biologics which reduces product development risks for our development partners and for our CMO clients.
www.shire.at

Genethon, located in Evry, France, is a non-profit R&D organization dedicated to the design and development of gene therapy treatments for rare genetic diseases from research to clinical validation. It was created by the AFM-Telethon (French Muscular Dystrophy Association) which is its main funding source.

Genethon has multiple ongoing programs at clinical, preclinical and research stage for neuromuscular, blood, immune system, and liver diseases.

Several of these programs are pursued by Genethon as sponsor, others have been licensed to leading biotech and pharma players in the gene therapy arena such as Audentes, Avexis/Novartis, Gensight Biologics, Orchard Therapeutics, Spark. Genethon was awarded the "Prix Galien France" in 2012..
www.genethon.fr

PARTNERS

GOLD

Human Gene Therapy is the premier peer-reviewed journal in the field and includes HGT Methods and HGT Clinical Development, providing 22 issues of comprehensive, end-to-end coverage that is driving today's explosion of gene therapy advances. Human Gene Therapy serves as the Official Journal of ESGCT. Come visit us at booth 31 to pick up your copy of the special issue dedicated to stem cell gene therapy, and meet Graham Parker, PhD, the Editor-in-Chief of Stem Cells and Development, a premier source of clinical, basic, and translational research on stem cells of all tissue types and their potential therapeutic applications. Stem Cells and Development is an Official Journal of the British Society for Gene and Cell Therapy.

Mary Ann Liebert, Inc. publishers is pleased to announce the 2018 launch of The CRISPR Journal, stop by booth 31 or visit www.liebertpub.com/CRISPR for information.
www.liebertpub.com/overview/human-gene-therapy

KCT is newly formed research center in Kuopio, Finland. We provide high quality basic and translational research. Professional team and laboratories with cutting edge devices are providing scientific know-how and modern technologies to develop advanced therapies. We undertake science in collaboration with academic groups all over the world. Link to FinVector's GMP manufacturing, quality and regulatory teams ensures product path from research to patients. Currently KCT is searching for new projects on gene- and cell therapy. Come to visit our stand!
www.kct.fi

MaxCyte is a global cell-based medicines and life sciences company applying its patented cell engineering technology to help patients with high unmet medical needs in a broad range of conditions. The company leverages its Flow Electroporation® Technology to enable its partners across the biopharmaceutical industry to advance the development of innovative medicines, particularly in cell therapy, including gene editing and immuno-oncology. MaxCyte has placed its cutting-edge flow electroporation instruments worldwide, including with nine of the top 10 global biopharmaceutical companies, and has more than 55 partnered program licenses in cell therapy including more than 25 licensed for clinical use. With its robust delivery technology, MaxCyte helps its partners to unlock the full potential of their products.

For more information, **www.maxcyte.com**

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PARTNERS

GOLD

MolMed S.p.A. is a medical biotechnology company focused on research, development and clinical validation of novel anticancer therapies. MolMed's pipeline includes antitumour therapeutics in clinical and preclinical development: Zalmoxis® (TK), a cell-based therapy; NGR-hTNF, a novel therapeutic agent for solid tumours; CAR-CD44v6, an immuno-gene therapy project; MolMed also conducts cell and gene therapy projects in collaboration with third parties, offering resources and expertise covering preclinical to Phase III trials activities. MolMed is listed on the main market (MTA) of the Milan stock exchange managed by Borsa Italiana.
www.molmed.com

Synthego is a leading provider of genome engineering solutions. The company's product portfolio includes software and synthetic RNA kits designed for CRISPR genome editing and research. With next-generation informatics and machine learning, Synthego's vision is to bring precision and automation to genome engineering, enabling rapid and cost-effective research with consistent results for every scientist.
www.synthego.com

Voyager Therapeutics is a clinical-stage gene therapy company focused on developing life-changing treatments for severe neurological diseases. Voyager is committed to advancing the field of AAV gene therapy through innovation and investment in vector engineering and optimization, manufacturing and dosing and delivery techniques. Voyager's pipeline focuses on severe neurological diseases in need of effective new therapies, including Parkinson's disease, a monogenic form of ALS called SOD1, Huntington's disease, Friedreich's ataxia, neurodegenerative diseases related to defective or excess aggregation of tau protein in the brain including Alzheimer's disease and severe, chronic pain. Voyager has broad strategic collaborations with Sanofi Genzyme, the specialty care global business unit of Sanofi, AbbVie, and the University of Massachusetts Medical School. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager Therapeutics is headquartered in Cambridge, Massachusetts.
www.voyagertherapeutics.com

PARTNERS

SILVER

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.
www.abeonatherapeutics.com

AFM (French Muscular Dystrophy Association) has a single objective: to defeat neuromuscular diseases, which are devastating muscle-wasting diseases. Created in 1958 by a group of patients and their families, and recognised as being of public utility in 1976, it has set itself two missions: curing neuromuscular diseases and reducing the disabilities they cause.
www.afm-france.org

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

BioNTech Innovative Manufacturing Services – Experts in Cell and Gene Therapy Manufacturing
BioNTech Innovative Manufacturing Services (IMFS) is a German Contract Development and Manufacturing Organisation specialized in the industrialization of cell and gene therapy products (viral vectors, cells and ivt mRNA). Based on extensive expertise in scientific, technical and regulatory prerequisites, we develop and manufacture your products in a safe and cost-efficient way in our state-of-the-art GMP facility. We offer a complete service spectrum from process development through clinical trial to in-market supply. All services are fully integrated and supervised by our QA department ensuring efficient and compliant manufacturing in our state-of-the-art GMP facility.
www.biontech-imfs.de

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PARTNERS

SILVER

Delphi Genetics, located in Belgium, is a biotechnology company specialized in genetic engineering with a deep expertise in DNA and its applications. Certified GMP we provide technological and flexible biomanufacturing solutions.

Delphi Genetics is involved in several DNA vaccines and gene therapy programs, including the development of CAR-T and collaborates on projects at all stages of development, from R & D to clinical phases. Our complete, GMP-compliant, flexible and single-use process for plasmid DNA manufacturing allows us to provide R&D, High Quality and GMP grade plasmid DNA from a few mg up to several grams in our dedicated facility.

To support this manufacturing activity, Delphi Genetics also offers a proprietary "antibiotic-free" technology, STABY®. The STABY® technology is implementable in all plasmid DNA and provide a better plasmid stabilization, a production yield increase and a safer way to manufacture plasmid DNA without antibiotic resistance gene following regulatory agencies recommendations.
www.delphigenetics.com

A pioneer in scholarly open access publishing, MDPI has supported academic communities since 1996. Based in Basel, Switzerland, MDPI has the mission to foster open scientific exchange in all forms, across all disciplines. Our 212 diverse, peer-reviewed, open access journals are supported by over 35,500 academic editors. We serve scholars from around the world to ensure the latest research is freely available and all content is distributed under a Creative Commons Attribution License (CC BY).
www.mdpi.com

Nature Technology Corporation (NTC) partners with biopharmaceutical and drug development companies to produce safe and effective non-viral vectors incorporating the best-in-class minimal Nanoplasmid TM vector backbone. Nanoplasmid TM vectors comprise a regulatory compliant antibiotic free selection marker (RNA-OUT) combined with a high-manufacturing-yield, host-restricted mini-origin of replication to deliver superior expression level and duration,and reduced transfection associated toxicity. Nanoplasmid TM vectors are ideal for gene and stem cell therapies, and as helper and packaging vectors for viral vector manufacture. NTC provides outsourcing for custom vector design and synthesis, and plasmid manufacturing, using its leading HyperGRO TM process, resulting in rapid development of gene and stem cell therapeutics and DNA vaccines.
www.natx.com

PARTNERS

SILVER

Novasep is a CDMO specialized in viruses & viral vectors production for tox, clinical and commercial phases. Novasep offers global and integrated services from process development to commercial manufacturing. For more than 15 years, we have acquired experience in manufacturing viral vectors (lentivirus, AAV, ADV...) for gene therapy and vaccination. Our investment in new commercial assets, addressing both drug substance and drug product manufacturing processes, can help to contribute to your project's success. Come and meet us at booth 33.
www.novasep.com

Orchard Therapeutics is a fully integrated commercial-stage biotechnology company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies.
Orchard's portfolio of autologous *ex vivo* gene therapies includes Strimvelis, the first autologous *ex vivo* gene therapy approved by the EMA for adenosine deaminase severe combined immunodeficiency (ADA-SCID). Additional programs for primary immune deficiencies, inherited metabolic disorders and blood disorders include three advanced registrational studies for ADA-SCID, metachromatic leukodystrophy (MLD) and Wiskott-Aldrich syndrome (WAS), clinical programs for X-linked chronic granulomatous disease (X-CGD) and beta-thalassemia, as well as an extensive preclinical pipeline.
Orchard currently has offices in the U.K. and the U.S., including London, San Francisco and Boston.
www.orchard-tx.com

Paragon Bioservices is an award-winning Contract Development & Manufacturing Organization (CDMO). Our aim is to build strong client partnerships focusing on transformative technologies, including oncology, immunotherapies (CAR-T cell therapies and oncolytic viruses), new generation vaccines (VLPs) and gene therapies (AAV). Paragon's cGMP facilities include microbial and mammalian suites, aseptic fil-finish and full-segregated virus facilities.
Responsibility to our clients, a passion for science and our collective need to contribute to better public heath—is what keeps us motivated and excited about the work that we perform. We are driven by our commitment to provide exceptional quality scientific performance and customer service.
With over 25 years in the business, Paragon's scientists, engineers, quality systems personnel and project managers have many years of experience working with biologics—from research and process development services to GMP manufacturing for clinical trials and eventual commercial launch.
www.paragonbioservices.com/therapeutic-areas/gene-therapy/

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PARTNERS

SILVER

Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. Sangamo is conducting Phase 1/2 clinical trials in Hemophilia A and Hemophilia B, lysosomal storage disorders MPS I and MPS II, and beta thalassemia. Sangamo has an exclusive, global collaboration and license agreement with Pfizer Inc. for gene therapy programs for Hemophilia A and gene regulation programs for C9ORF72-linked amyotrophic lateral sclerosis and frontotemporal lobar degeneration; with Kite, a Gilead Company, for next-generation autologous and allogeneic engineered cell therapies for the treatment of cancer; with Bioverativ, a Sanofi Company, for hemoglobinopathies, including beta thalassemia and sickle cell disease; and with Shire International GmbH to develop therapeutics for Huntington's disease. **www.sangamo.com**

Selecta Biosciences, Inc. is a clinical-stage biopharmaceutical company that is focused on unlocking the full potential of biologic therapies by mitigating unwanted immune responses. Selecta plans to combine its tolerogenic Synthetic Vaccine Particles (SVP™) to a range of biologics for rare and serious diseases that require new treatment options. The company's current proprietary pipeline includes SVP-enabled enzyme, oncology and gene therapies. SEL-212, the company's lead candidate in Phase 2, is being developed to treat severe gout patients and resolve their debilitating symptoms, including flares and gouty arthritis. A Phase 1 trial is ongoing for a combination therapy consisting of SVP-Rapamycin and LMB-100 (Selecta's SEL-403 product candidate) for the treatment of patients with malignant pleural or peritoneal mesothelioma. Selecta's proprietary gene therapy product candidates are being developed for rare inborn errors of metabolism and have the potential to enable repeat administration. The use of SVP also holds potential in the development of vaccines and treatments for allergies and autoimmune diseases. **www.selectabio.com**

uniQure is delivering on the promise of gene therapy, single treatments with potentially curative results. We have developed a modular technology platform to rapidly bring new disease-modifying therapies to patients with CNS, liver/metabolic and cardiovascular diseases. We are advancing a focused pipeline of innovative gene therapies and have entered late-stage clinical development in our lead indication, hemophilia B, and established preclinical proof-of-concept in Huntington's disease. **www.uniqure.com**

PARTNERS

SILVER

Yposkesi is the largest European CDMO for gene therapy vector manufacturing. Created in November 2016 in Corbeil Essonnes (France) as a spin off from the world-class gene therapy pioneer Genethon, Yposkesi provides a full-service offer covering BioProcess development (USP & DSP), from small/pilot to large production scale,analytical development, GMP manufacturing of clinical lots of lentiviral vectors and regulatory support.
Its current facility consists in a 50,000 sq ft building, operating 4 manufacturing suites for bulk drug substance, and 2 Fill & Finish suites. This capacity will be expanded in 2021 with a large scale facility (50,000 sq ft) equipped with 2,000L bioreactors, designed for commercial production and compliant to EMA and FDA. Capitalizing on more than 25 years of expertise, Yposkesi significantly invests in innovation in bioprocessing to cost-effectively deliver on high quality projects.
www.yposkesi.com

BRONZE

Anemocyte is a Biotech Manufacturing Organization (BMO): a biotech company that addresses CGT needs pro-actively offering one stop shop solutions and fostering exciting innovations active in the field of Cell and Gene Therapies (CGTs).
Our experience and know-how come from far:

- 14 years of GMP manufacturing of CGTs and biological drugs;
- 61 years of Contract Manufacturing within our Group (Holding F.I.S.)

Our business includes:

- Process development and GMP Manufacturing (Somatic Cells, Non-Viral Modified Cells and Extracellular vesicles)
- Plasmid for Viral Vector Manufacturing
- Project 2020: up to commercial manufacturing capabilities (Including use of Viral Vectors for CGTs)

www.anemocyte.com

Axon Medchem is a trusted supplier of high-value life science products, providing Axon Ligands™ as world wide recognized drug reference standards for pharmacological research. With more than 2000 excellent quality small molecule inhibitors and modulators targeting more than 850 biological targets, we aim to facilitate your scientific research and development.
Axon Medchem is also a leading European CRO in medicinal chemistry, specialized in contract research and high-quality synthesis of bio-active and/or drug-like molecules. We have the proven record in developing novel drug candidates and achieving excellence for a decade by providing our dedicated chemistry services for companies and research institutes around the world.
www.axonmedchem.com

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Cell Press is a leading publisher of biomedical and physical science research, offering high-quality, cutting-edge research and resources. We drive science forward and promote cross-pollination of ideas with our passion for excellence and commitment to innovation. Our aim is to engage the scientific community by communicating important, exciting discoveries made today that will impact the future of research. We are also proud to publish Stem Cell Reports, the official journal of the ISSCR, and the Molecular Therapy family of journals, the portfolio of gene and cell therapy research journals for the ASGCT.
www.cell.com

Generation Bio aims to do what no other gene therapy platform has yet accomplished: to deliver titratable, re-dosable genetic medicines with drug-like properties to patients with a wide range of genetic diseases.
www.generationbio.com

The Ontario Institute for Regenerative Medicine (OIRM) is a non-profit stem cell institute funded by the Ontario government and dedicated to transforming discoveries into clinical trials and cures. Through our commitment to collaboration and partnerships, we leverage our resources to fund and support promising advances. OIRM is a passionate champion for investigators and their patients as we build a healthier future for Ontario, Canada, and the world.
www.oirm.ca

TiGenix is an advanced biopharmaceutical company developing novel therapies for serious medical conditions by exploiting the anti-inflammatory properties of allogeneic, or donor-derived, stem cells. TiGenix´ lead product, Alofisel (darvadstrocel), previously Cx601, received European Commission (EC) approval for the treatment of complex perianal fistulas in adult patients with non-active/mildly active luminal Crohn's disease, when fistulas have shown an inadequate response to at least one conventional or biologic therapy. A global Phase III trial intended to support a future U.S. Biologic License Application (BLA) started in 2017. TiGenix has recently been acquired by Takeda, a global pharmaceutical company active in gastroenterology.
www.tigenix.com

ESGCT SPRING SCHOOL 2019

EUROPEAN SOCIETY OF GENE & CELL THERAPY

Book from 1 December 2018 to secure one of 150 seats.

Naples, Italy, 3-5 April

An intensive three-day training course for PhD students and early career researchers new to gene and cell therapy

- The opportunity to network with Europe's current and future leaders in the field
- Support for early career researchers
- Debates
- Workshops: Public engagement | How to publish a paper | Presentation skills

150 spaces only | free registration for students

Session themes

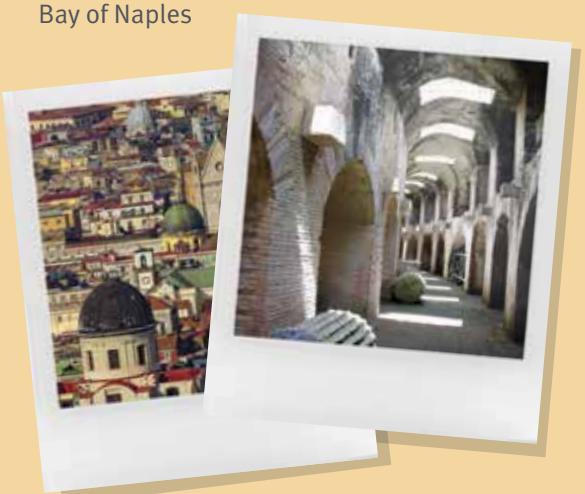
Spotlight on delivery tools | Spotlight on stem cells | Gene and cell therapy for eye diseases | Genome editing and engineering | Gene and cell therapy for liver diseases | Gene and cell therapy for CNS | Immunotherapy and cancer | Gene and cell therapy for PID

Some of the confirmed speakers

Alessandro Aiuti | Robin Ali | Alberto Auricchio | Andrea Ballabio | Chiara Bonini | Juan Bueren | Hildegard Büning | Nathalie Cartier | Anna Cereseto | Vincenzo Cerullo | Lucia Faccio | Antonella Folgori | Alessandro Fraldi | Petro Genovese | Nicola Brunetti Pierri | Anne Galy | Guangping Gao | Pietro Genovese | Angelo Lombardo | Federico Mingozzi | Luigi Naldini | Axel Schambach | Guiseppe Testa | Adrian Thrasher

Fun and social

Visit the sunken city of Pozzuoli | Meander in the streets of Naples | Eat a real Neapolitan pizza overlooking the Bay of Naples



2019

www.esgct.eu/Congress/Spring-School.aspx

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Booth 52: Ajinomoto Co., INC. is one of the largest amino acid companies in Japan and is now focusing on cell therapy and stem cell research. We provide stem cell media and growth factors of clinical grade.
www.ajinomoto.com

Booth 40: Aldevron serves the biotechnology industry with custom production of nucleic acids, proteins and antibodies. Featuring the largest and most modern GMP plasmid DNA manufacturing facility in the world, Aldevron provides thousands of clients with plasmids, RNA, gene editing enzymes and more for biological research projects from discovery to clinical trials and commercial applications.

These products are critical raw materials and key components in commercially available drugs and medical devices, helping accelerate development of treatments for diseases such as cancer, infectious disease, pediatric disorders and rare diseases.

Our collaborative approach and commitment to providing quality materials allow us to meet precise client requirements and provide innovative solutions to advance science. Aldevron headquarters is in Fargo, North Dakota USA, and has facilities in Madison, Wisconsin and Freiburg, Germany.
www.aldevron.com

Booth 38: ALS CellCelectorTM is the only system which enables automated isolation of single cells, clusters, adherent cell colonies or colonies grown in 3D semi-solid media. It's an ideal system for (i) automated clonal picking of newly derived iPSC colonies, (ii) single cell or colony isolation for genome editing, and (iii) automated picking of hematopoietic stem cell colonies. Isolated colonies or single cells can be deposited into a variety of destination plates for downstream culturing or molecular characterisation (qPCR, sequencing...). CellCelector combines bright field, phase contract or fluorescence imaging, sensitive cell/colony detection technology and patented robotics picking tools. The system can be also used for stem cell culture monitoring and be integrated into a fully automated stem cell production facility.
www.als-jena.com

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Booth 13: The American Society of Gene & Cell Therapy (ASGCT) is the primary membership organization for scientists, physicians, professionals, and patient advocates involved in gene and cell therapy. Our mission is to advance knowledge, awareness, and education leading to discovery and clinical application of gene and cell therapies to alleviate disease—we do that by serving as a catalyst to transform medicine by advancing gene and cell therapies to benefit patients and society. ASGCT's programs include an annual scientific meeting in the United States each spring welcoming more than 3,000 people as well as publication of the *Molecular Therapy* family of four journals. **www.cell.com/molecular-therapy-family/home**

Booth 5: ArcticZymes has a rich history leveraging its access to the marine Arctic; identify novel cold-adapted enzymes for use in molecular research, diagnostics, and bioprocessing. ArcticZymes' products are firmly entrenched in the molecular research and diagnostic arena; as both standalone enzymes and unique components in the kits of our commercial partners. We have developed strong relationships with OEM partners and individual researchers alike with the strategic intent to expand our enzyme portfolio and the application of each unique product. ArcticZymes has recently launched a novel Salt Active Nuclease that is successfully being used by producers of viral vaccines in gene therapy. This nonspecific, recombinant endonuclease has optimum activity at high salt concentrations, which can improve efficiency and yield in various workflows.
www.arcticzymes.com

Booth 48: Booth 48: ASEPTIC TECHNOLOGIES provides the technology for cGMP aseptic fill and finish for ATMP (AT-Closed Vial® Technology), combining:

- The Closed Vial, ensuring 100% of Container Closure Integrity during cryostorage and ultimate sterility assurance. Supplied ready-to-fill and remaining closed through all process.
- The Filling Equipment, robust and scalable solution, performing only 3 operations: filling by piercing, sealing and capping. Available in manual and automated modifications.

Our technology is widely used for cGMP filling of ATMPs for years, since it enables maximum safety during filling, cryostorage, shipping and administration; allows minimizing the Cost of Goods and an easy scaling-up.
AT-Closed Vial® is compatible with liquid nitrogen-free controlled rate freezers by GE Asymptote. Automated thawing device is available for standardization of the administration. **www.aseptictech.com**

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Booth 39: Asphalion is an international Scientific and Regulatory Affairs consultancy, with offices in Barcelona, Madrid and Munich. We collaborate with Pharma and Biotech companies facilitating Drug Development and Regulatory Affairs projects for Drugs, Biologics, Biosimilars, ATMPs and Medical Devices. Our involvement ranges from early development, through to registration and post-commercialization phases. Since the company was founded in 2000, we have consistently grown and now have a team of over 80 employees with backgrounds in all areas of life sciences. Our consultants are experts in their fields and are in direct contact with European agencies for the implementation of new regulatory standards. We provide global services and work for hundreds of clients from around the world. Through collaborations with partners in all other continents, we can accelerate your worldwide scientific and regulatory activities by using local expertise.
Services:

- Regulatory and Scientific Strategy during Development
- Medical and Scientific Writing
- Global Submissions
- eSubmission and RIM
- Life-Cycle Outsourcing
- Pharmacovigilance
- Promoting the marketing registration of Medical Devices: Classification strategy, CE Marking and compilation of technical file.

www.asphalion.com

Booth 59: AWST linear scalable, continuous flow, ultracentrifugation solutions reliably and efficiently separate, purify, and concentrate viral vectors and virus like particles in the development and manufacture of vaccines and other bio-products. Successful AAV purification has been achieved with yields exceeding 55%, recovery rates to 50%, and dramatically reduced turnaround time as compared to conventional column purification. Systems are available for research, pilot scale production, and full scale manufacturing.
In addition to ultracentrifugation, AWST offers a fully automated system for fluid handling and fractionation in a self-contained, mobile work station. System design supports pinch valves for fluid routing, pumps for flow and direction, an inline refractometer for fractionation/monitoring and disposable flow, pressure and temperature transducers for process monitoring. With over 60 years of experience and service to the bioprocess community, AWST is the clear choice when looking for an experienced supplier.
www.awst.com

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Booth 55: AMSBIO, a global provider of advanced life science technology. Experts in making oncology assays more physiologically relevant, our products and services include antibodies, peptides and recombinant proteins, tools for spheroid and organoid culture as well as systems for studying cell motility, migration, invasion and proliferation. New cancer drug screening and profiling service also now available.
www.amsbio.com

Booth 62: BioLamina is a Swedish biotechnology company with a global presence. We offer an expansive portfolio of defined human recombinant laminin proteins, Biolaminins, for a variety of applications, including reliable expansion of human pluripotent stem cells and differentiation and maintenance of different specialised cell types, such as hepatocytes, cardiomyocytes and neural cells. By providing the cells with their biologically relevant matrix proteins, the natural, cell-specific cell-matrix interaction can be mimicked, leading to improved cell functionality, robust culture systems and safe cells for therapy. BioLamina's laminin technology has been scientifically validated in many high impact journals.
www.biolamina.com

Booth 6: Founded in 1981, Biological Industries specializes in the development, manufacture, and distribution of IVD and research products for cell culture, focusing today on stem cell systems and media for cell therapy. BI offers a full range of xeno-free stem cell products and services, which includes stem cell culture media, freezing media, attachment factors, and cell dissociation solutions. BI products include: the Nutristem® range of cell media (for mesenchymal, induced pluripotent, and embryonic stem cells), and BIOTARGET™ (optimized specifically for activation and expansion of T cells), serum-free, xeno-free media for research and clinical applications, helping to advance stem-cell-based therapies. We are committed to a Culture of Excellence through our advanced manufacturing and quality-control systems, superior regulatory expertise, and extensive technical customer support, training, and R&D capabilities.
www.bioind.com

Booth 27: BioNTech
See page 54.
www.biontech.de

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Booth 17: 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 50: Bluebird Bio
See page 47
www.bluebirdbio.com

Booth 25: Brammer Bio
See page 50
www.brammerbio.com

Booth 24: Caltag Medsystems provide Life Science and Drug Discovery scientists with a portfolio of over 200,000 high quality research and diagnostic reagents across Europe. Our Cell Culture portfolio ranges from primary cells, stem cells, cell lines and media to skin, fresh animal brains, healthy and diseased products, angiogenesis models and transfection reagents.
Through our suppliers, we are able to offer:

- Human and Animal Tissue Sourcing Service
- Fresh and Frozen Bone Marrow
- CD34+ Cells (Cord Blood, Peripheral Blood, Mobilised Peripheral Blood, Bone Marrow)
- Leukapheresis Packs (mononuclear cells and autologous platelet rich plasma)
- Supplements and Media

We can source cells types according to specific donor requirements, such as age, sex etc. and there are a large number of cell types are available. With our fully customised tissue procurement service, we are able to source tissue with specific donor inclusion/exclusion criteria, all QC tested and pathology verified.
www.caltagmedsystems.co.uk

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Booth 12: CEVEC is a center of expertise for the production of biopharmaceuticals using a unique human cell-based production system. CAP-GT is a regulatory endorsed expression platform for scalable viral vector production. CEVEC has successfully developed CAP-GT suspension cell-derived viral packaging cell lines which enable better scale-up and competitive production costs when compared to adherent cell culture systems. CAP-GT suspension cell lines grow to high cell densities and show a broad viral propagation spectrum. Gene therapy vectors such as lentivirus (LV), adenovirus (AV) and adeno-associated virus (AAV) can be produced at industrial scale. **www.cevec.com**

Booth 23: ChemoMetec develops, manufactures and sells high quality automated Image Cytometer's within cell counters, which as the only ones on the market can count and analyse aggregated cells, adipose derived stem cells, cells growing on microcarriers with the highest precision. We also offer advanced cell analysers to help streamline processes for maximum efficiency. Our instruments are widely used in fields such as cancer research, stem cell research, production and quality control of a number of products such as pharmaceuticals, beer, animal semen and milk. We have specialised assays for aggregated cells, cells growing on microcarriers and adipose derived stem cells. 21 CFR Part 11 is also valued highly to have the highest standards. Our products are held in high regard because of their high quality and precision as well as the 'ease of use' advanced cell analysis. We value our customers; Therefore our policy is "no hidden costs" - no service agreements, high level of support and free software updates. **www.chemometec.com**

Booth 56: Over 5 years of partnership hundreds of GMP studies performed through an integrated management scheme for recombinant proteins, gene therapy, and vaccine products.
Clean Cells capabilities: GLP/GMP certification for Quality Control and manufacturing | Bacterial, Eukaryotic (producer or packaging cells)and Viral Bank GMP manufacturing | IMP Manufacturing (cell therapy products) | GMP storage | Microbiological and viral testing | Identity – Karyotype - FISH | Adventitious virus testing | Retrovirus detection | Specific contaminants detection (NAT methods) | Genetic characterization | Residual DNA/HCP quantitation | RcAAV & RCL
Genibet capabilities: GMP certification for APIs and IMPs Manufacturing | Biopharmaceuticals GMP Manufacturing (Drug Substance and Drug Product) | Bacterial and Yeasts Master/ Working Cell Bank GMP | Manufacturing | Eukaryotic Master /Working Cell Bank GMP Manufacturing | Master /Working Virus Seed Stock GMP Manufacturing | Cell and Gene Therapy GMP Manufacturing | Fill and Finish | Customized offer | Quality Assurance Services | High flexibility and versatile structure | Dedicated Project Manager and single contact point | Highly qualified team **www.clean-cells.com** **www.genibet.com**

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Booth 44: Delphi Genetics
See page 55
www.delphigenetics.com

Booth 7: In the early 80s' the establishment of CELBIO represented a true milestone, as this Company quickly became a reference for a number of scientists, giving them a valuable opportunity to gain access to a world of products and equipment in Biotechnology. Later on, this name became familiar also in the Medical and Diagnostic field. This leading approach remained unchanged and possibly improved throughout almost three decades, reinforcing the image of this Company as a reliable and solid partner for the entire scientific community. At the beginning of the 90s' a sister Company called Euroclone® started its activity with the goal of giving more emphasis to the development of smart ideas which were felt to evolve into nice products to sell. The success was such that in 2009 it was decided to merge the two Companies, and CELBIO was fully incorporated into Euroclone®. As a matter of fact, Euroclone® nowadays is bringing into the market the "passion" of the early days (since has inherited the Distribution of a panel of well known brands) along with a clear and growing interest in innovative and state-of-the art technologies originated by in-house R&D as well as obtained under private label and OEM agreement with the most qualified manufacturers worldwide. In other words, Euroclone® continues the tradition of CELBIO, while evolving into a modern supplier of up-to-date and own-branded products, thus reconfirming its position as a key-provider for a number of laboratories and clinics.
www.euroclonegroup.it

Booth 34: FinVector is a world leader in the research and development of Viral-Based Gene Therapy products, with state-of-the-art facilities and a highly experienced scientific team working in the gene therapy market. We deliver a tailored service to meet and exceed our clients' needs, and use our scientific expertise and industry knowledge to help clients take viral-based products from the pre-clinical phase, through clinical trials and to the market. Visit us on our stand!
www.finvector.com

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Booth 8: Flash Therapeutics is a new gene therapy company developing gene and cell-based therapies leveraging its proprietary lentiviral production platform. The Company is built around the Vectalys lentiviral platform, which includes its patented non-integrative LentiFlash® technology, and integrative lentiviral vectors. By providing efficient, transient and short-term RNA delivery, LentiFlash is suitable for gene editing and other advanced therapeutic approaches. When stable DNA expression is needed (immunotherapies such as CAR T cells), integrative lentiviral vectors will be the delivery method of choice. Both technologies benefit from novel production and purification processes developed and continually optimized since 2005. Flash Therapeutics is advancing two business lines: therapeutic development based on LentiFlash, with internal programs for blood and liver diseases; development and manufacturing support to companies worldwide pursuing lentiviral-based therapies. Flash Therapeutics was established in 2018 through the merger of FlashCell, a gene therapy company developing LentiFlash®; and Vectalys, a 13-year old lentiviral vector manufacturer.
www.flashtherapeutics.com

Booth 15: Gene Therapy Center Austria
See page 51
www.shire.at

Booth 4: GeneWerk GmbH is a German startup company. The team has long-lasting experience in the area of hematology, oncology and virology with focus on integration site analysis, sequencing and bioinformatics. GeneWerk provides quality controlled custom-tailored service based on 20 years of experience in the field of gene therapy, gene editing, immunotherapy and related areas.
www.genewerk.de

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Booth 37: GenoSafe is a CRO providing analytical testing services under GLP, GMP and GCP compliance. We help our clients through their drug development process by caring out the laboratory evaluation of the quality, safety and efficacy of their innovative products (AAV, Lentivirus, Retrovirus, CAR-T cell, Stem Cells).

Working for the leaders in Gene and Cell therapy, GenoSafe has been involved in immunogenicity studies, biodistribution-biodissemination studies and manufactured batches characterization.

Our proudest achievement was to be able to assist our clients from preclinical research to clinical studies and Quality Control testing.

We have a customizable approach with our clients, using our expertise to set up the right method testing on their product that will go all the way through the Drug Application
www.genosafe.com

Booth 31: Human Gene Therapy
See page 52
www.liebertpub.com/overview/human-gene-therapy

Booth 57: Environmental Risk Assessment Services Since 2001, Jenal & Partners Biosafety Consulting provide Environmental Risk Assessments and Biologics Assessment of gene and cell therapy products as well as recombinant vaccines and oncolytic viruses for both clinical trials and market authorisation applications submitted to EMA and FDA, respectively. One of the first hour ERAs has been the one for Glybera by UniQure. Jenal & Partners have extensive experience and background knowledge in the environmental risk assessment of gene and cell therapies involving AAV, Adeno-, Lenti- and Retrovirus, HSV, MVA, NYVAC, VSV... Our clients are both small local and large international pharmaceutical companies. In the context of the ERA design, services include responding to questions from competent authorities, compilation of ATMP safety data sheets, definition of ATMP safe administration procedures and safety training of medical personnel.
www.jenalpartners.ch

Booth 35: KCT
See page 52
www.kct.fi

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Booth 30: Lonza
See page 48
www.lonza.com

Booth 43: MaxCyte
See page 52
www.maxcyte.com

Booth 36: MDPI
See page 55
www.mdpi.com

Booth 42: BioReliance | Merck
See page 46
www.bioreliance.com

Booth 16: 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. Used by scientists and clinicians around the world, our technologies cover techniques of sample preparation, cell isolation, cell sorting, flow cytometry, and cell culture. Our more than 25 years of expertise spans research areas including immunology, stem cell biology, neuroscience, and cancer. Today, Miltenyi Biotec has more than 2,000 employees in 28 countries – all dedicated to helping researchers and clinicians make a greater impact on science and health.
www.miltenyibiotec.com

Booth 32: Molmed
See page 53
www.molmed.com

Booth 58: Nature Technology
See page 55
www.natx.com

Booth 9: NHS Blood and Transplant is a national organisation within the NHS dedicated to saving and improving lives. Our Cellular and Molecular Therapies function offers broad experience and expertise in the provision and manufacture of cell and gene therapies through our national network of GMP grade and MHRA licensed facilities. NHSBT partner with academic, commercial and NHS organisations supporting novel cell and gene therapy programmes from concept through to clinical trial, operating a 'not for profit' model. We welcome expressions of interest for partnerships with organisations striving to develop the future of medicine in the advanced therapies space.
www.nhsbt.nhs.uk

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Booth 33: Novasep
See page 56
www.novasep.com

Booth 53: We focus and are highly specialized on applications in cell culture, stem cell expansion and differentiation, cell counting, sample preparation and cell assays. We continuously monitor the world's most innovative life science markets to expand our portfolio for your benefit. Visit our homepage www.ols-bio.ch for an overview. With our customer-centric philosophy, we provide comprehensive support and advice before and after instrument installation. In fact, this is the starting point of a valuable relationship for the benefit of our customers. For us, application support as well as service and maintenance of the systems is highly important and our daily business. We maintain a close contact with the manufacturers of our systems and our application specialists are continuously trained and kept up-to-date. This ensures optimal support with respect to all of your technical and applicative needs. Any of our system is available for demonstration in your laboratory. Get in touch with us, we look forward to meeting you!
www.ols-bio.ch

Booth 54: Orchard Therapeutics
See page 56
www.orchard-tx.com

Booth 26: Oxford BioMedica
See page 46
www.oxfordbiomedica.co.uk

Booth 61: Oxford Genetics is a leading synthetic biology company focused on developing novel technologies to overcome the challenges associated with the discovery, development and production of biologics, gene therapies, cell therapies and vaccines. Our proprietary genomic system enables the precise engineering of DNA, which through automated production platforms, analytical assessment and process development, drive the rational design of complex biological systems. Taking this approach we have developed several proprietary platform technologies, including:

- Mammalian cell display system and humanization algorithm for the discovery and engineering of antibodies
- Viral vectors for transient expression of AAV and lentivirus
- Viral packaging and production cell lines for stable lentivirus and AAV manufacture
- CRISPR pooled and arrayed libraries
- CRISPR cell line engineering

www.oxfordgenetics.com

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Booth 29: Pall Biotech provides cutting-edge products and services to meet the demanding needs of customers discovering, developing and producing biologics and classic pharmaceuticals. The company's membranes and membrane devices optimize detection and sample preparation in the drug research, clinical diagnostics, genomics, and proteomics markets. Pall is a leading provider of automated systems and single-use solutions to pharmaceutical and biotechnology companies - from upstream, through downstream, to formulation and filling - and maintains certified ISO9001 manufacturing facilities worldwide. The company's Scientific and Laboratory Services (SLS), Technical Services and Validation Laboratories have been a cornerstone of customer support for more than 30 years providing compatibility studies, extractable/leachable studies, particulate validation and more.
www.pall.com/biotech

Booth 45: Paragon
See page 56
www.paragonbioservices.com/therapeutic-areas/gene-therapy/

Booth 41: PathoQuest, a leader in next-generation sequencing (NGS) based adventitious virus testing, is offering a game-changing NGS-based metagenomics solution for Infectious Diseases Diagnostics and Biologics Genomic Services.

PathoQuest's metagenomics NGS service is a fast, cost-effective and fully integrated high-throughput sequencing option for viral safety testing that enables the ability to do a single test instead of multiple assays. PathoQuests's proprietary sample-to-report process will help you to address the current limitations associated with pathogen detection across numerous biological samples types. In addition to viral safety assessment, we offer microorganisms sequencing and genetic cell bank characterization. Meet our expert Jean-Marie Charpin Ph.D., Chief Commercial Officer, Biologics Genomic Service, and his team at stand #41 to learn more about the expertise we can bring to your testing needs, our advanced bioinformatic platform and our state-of-the-art lab which meets the highest pharmaceutical industry quality standards.
www.pathoquest.com

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Booth 10: Supporting life science research since 1988, PeproTech is a privately owned biotechnology company focusing on the development and manufacture of high quality cytokine products for the life-science and cell therapy markets. Over the past 30 years the company has grown into a global enterprise with state-of-the-art manufacturing facilities in the US, and offices around the world. Our mission is to provide the highest quality products that address the needs of today's scientists and researchers, and we pride ourselves on being a trusted partner within the scientific community. With over 2,000 products PeproTech has developed and refined innovative protocols to ensure quality, reliability and consistency.
Our product range includes:
Research Grade Proteins and Antibodies | GMP-Compliant products for Cell, Gene and Tissue Therapy | Animal Free Cytokine Range | ELISA kits | Cell Culture Media Kits / Supplements
Please contact PeproTech to discuss your research requirements:+ 44 (0)20 7610 3062 **www.peprotech.com**

Booth 1: PlasmidFactory GmbH & Co. KG is a globally active biopharmaceutical company, founded in Bielefeld, Germany, in 2000. As a leading contract manufacturing organization (CMO) for plasmid and minicircle DNA, it has a strong customer base in the fields of cancer research, gene and cell therapy, CAR-T-cell development, and vaccination. PlasmidFactory produces plasmids and minicircles according to customer's requirements in modern laboratories with high quality standards. In addition to its In Stock products, e.g. for the worldwide exclusively offered pDG/pDP AAV 2-plasmid-system, and custom manufacturing of plasmid and minicircle DNA, PlasmidFactory focuses its R&D efforts on its core competencies in the production, analysis, application, and storage of DNA.
www.plasmidfactory.com

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Booth 28: Polyplus-transfection applies its 15+ year expertise to the development of novel transfection solutions for mammalian cells. We provide reagents for high yield transient protein and antibody production in CHO and HEK-293 cells, as well as for viral vector production for Gene and Cell Therapy (PEIpro product range). Our products meet the quality requirements for use in bioprocesses up to GMP grade.
www.polyplus-transfection.com

Booth 3: PROGEN, founded in 1983 and located in Heidelberg, Germany is an established manufacturer and supplier of AAV Titration ELISA tests and antibodies for gene therapy research and development. In the past 20 years, the DIN EN ISO 13485 certified company has established a unique portfolio of AAV tools for basic and clinical research as well as for pharmaceutical applications. Among them are specific antibodies to study AAV assembly, capsid formation or the course of an infection as well as a line of reliable AAV quantification ELISAs for different serotypes utilizing PROGEN's portfolio of capsid-specific AAV antibodies. PROGEN offers its broad expertise in antibody and ELISA technologies, protein interaction and purification and welcomes collaborations with academic and industrial institutions to advance basic AAV research. In addition, the company aims to establish partnerships with academic, pharmaceutical or medical institutions for the development of AAV-based tools and therapies, e.g. for standardization and validation steps in clinical trials.
www.progen.com

Booth 60: RoweMed AG – Medical 4 Life is an innovative MedTech company. Our focus is on developing and producing customized medical plastic systems, especially for the handling of sensitive pharmaceuticals.
R&D: The development of systems is closely coordinated with the customer, from first sketch up to a market-ready product, including CAD construction, prototyping, first series and product tests.
Production: We offer injection molding, assembling and packaging under one-roof. In the cleanroom production facility (ISO class 7) in Parchim our employees carry out single-part, small series and automated mass production. At the assembling department all common welding and gluing technologies are implemented.
Regulatory Affairs: In addition to the technical services we offer the complete technical documentation according to the requirements of the Medical Device Directive 93/42/EEC.
www.rowemed.de

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Booth 46: Sarepta Therapeutics
See page 49
www.sarepta.com

Booth 64: Sartorius Stedim – Regenerative Medicine
Sartorius Stedim Biotech is a global solution provider to the biologics industry and is well positioned to support regenerative medicine companies by applying technologies adapted for these sectors. With a broad range of unique and innovative technologies and services, Sartorius supports the development, analysis and manufacture of a range of different types of regenerative medicines with single-use automated systems. **www.sartorius.com**

Booth 2: SIRION Biotech is a world leader in innovating virus vector technologies and provides custom services to academic and industrial partners worldwide. SIRION is the only company mastering all 3 major virus types (LV, AV and AAV) that are used regularly for genetic manipulation of cell systems, whether for in vitro or *in vivo* purposes. Within the biotechnology sector SIRION cater to a number of research and development projects, most prominently in therapy development, e.g. gene and CAR-T cell therapy fields. For development of new cancer immunotherapies, SIRION Biotech has developed a universal adjuvant – LentiBOOST™ that can increase T- and B-cell transduction significantly. SIRION offers full customized services for gene therapy R&D by optimizing AAV tissue specificity and improving the immunogenic profile. SIRION's project oriented focus on optimization of vector technologies makes us the perfect partner to strengthen your R&D of new therapies.
www.sirion-biotech.com

Booth 14: STEMCELL Technologies Inc. is a Canadian biotechnology company that develops specialty cell culture media, cell isolation systems and accessory products for life science research. Our specialty media include cGMP grade mTeSR™1 and 3D culture kits, supporting the transition from bench to bedside for cancer research, cell therapy and regenerative medicine research, immunotherapy and more. Rigorous quality control processes and raw material selection are critical to the optimal performance of STEMCELL's entire portfolio of products, ensuring that batch-to-batch variability is minimized. Driven by science and a passion for quality, STEMCELL delivers over 2500 products worldwide with consistent, unfailing quality and one-on-one assistance to help you succeed. If there is a product or service that we do not currently offer, we are happy to work with you to customize media or cell isolation products to meet your specific needs.
www.stemcell.com

EXHIBITORS

EXHIBITORS

Booth 21: SYNENTEC provides tailored solutions to solve your individual cell culture challenges, like the proof of monoclonality for regulatory approval of cell lines, or viability assays and cell density measurements in process development. We develop automated cell culture microscopes such as CELLAVISTA and NYONE to offer a way to answer your individual cell culture questions in a fast and easy way. Our YT image analysis software is constantly being expanded with new applications and our products are used in the fields of cell line development, stemcell research, immunology, cell and gene therapy as well as in scientific research. Applications like... 1) Single Cell Cloning (SCC), High Resolution Cloning as well as Fluorescence activated SCC (FASCC), 2) Trypan Blue Viability Count, 3) Suspension Cell Count, Confluence Measurement and Colony Detection, 4) FASC Seeding Control, 5) Transfection Efficiency ...provide only a part of all the possibilities amongst which we facilitate your daily lab use and make it a little more professional! You like going more into the depth? Discover your cells with applications like... 1) Cell Nuclei Count and Characterization, 2) Immuno-stainings, 3) Antibody Internalization, 4) CD-marker Analysis, 5) iPS-cell Detection, 6) focus forming assays, 7) Toxicity Studies, 8) Apoptosis Monitoring, 9) Total Well Intensity Quantitation. The imagers support a wide range of cell culture systems in the SBS format, e.g. mikrotiter plates, transwell plates and microscope slides as well as culture dishes and T-Flasks.
www.synentec.com

Booth 18: Synthace is a London-based biotech/software company whose aim is to make life science research more efficient. Our sophisticated software allows scientists to easily and flexibly control their automation. This opens up the possibility for rapid DoE studies, media optimisation, assay setup, data analytics, and more.
www.synthace.com

EXHIBITORS

EXHIBITORS

Booth 11: Takara Bio Europe is a wholly owned subsidiary of Takara Bio Inc., who develops, manufactures, and distributes a wide range of life science reagents under the Takara™, Clontech®, and Cellartis® brands. Key products include SMART cDNA synthesis kits for a variety of samples and applications, including NGS, high-performance qPCR and PCR reagents (including the Takara Ex Taq®, Takara LA Taq®, Titanium®, and Advantage® enzymes), Cellartis stem cells and stem cell reagents, RT enzymes and SMART library construction kits, the innovative In-Fusion® cloning system, Guide-it gene editing tools, Tet-based inducible gene expression systems, and Living Colors® fluorescent proteins. Recently, Takara Bio acquired Rubicon Genomics and WaferGen Bio-systems. As part of the Takara Bio family, they expand our NGS portfolio as well as add R&D, manufacturing, and support for automation systems for NGS and qPCR applications. Takara Bio's portfolio supports applications including NGS, gene discovery, regulation, and function studies, as well as genetic analysis, protein expression and purification, gene editing, stem cell studies, and plant and food research.
www.takarabio.com

Booth 19: The Gene Therapy Program is a complex R&D organization with the internal capacity to perform gene transfer studies from basic research through phase 1/2 proof-of-concept human trials. We partner with industry leaders to advance the field of gene therapeutics. The Penn Vector core is a full service core with over a decade of experience in the production in viral-based vectors. The core's main objective is to provide investigators access to state-of-the-art vector technology for preclinical studies and other basic research applications.
www.gtp.med.upenn.edu

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COLLABORATIVE CONGRESS 2018

EXHIBITORS

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Booth 63: VectorBuilder, established in 2014, is a revolutionary online platform that allows researchers to design custom vectors/viruses and simply order them. We possess a massive collection of ORFs since we have generated over 165,000 vectors so far meaning we do not synthesize, which in turn means low cost. VectorBuilder in addition offers exceptionally high quality viruses, viral packaging, shRNA/gRNA CRISPR libraries and BAC modification as well as CRISPR/Cas9 transfection/microinjection kits.
www.vectorbuilder.com

Booth 20: Vironova is a Swedish biotechnology company providing comprehensive, hardware, software, and services for the analysis of nanoparticles. Vironova revolutionizes access to transmission electron microscopy–based image analysis in biopharmaceutical development. Our solution enables automated analyses for faster and better informed decisions to secure robust bioprocessing and final product quality.
www.vironova.com

Booth 22: VIVEbiotech is a company fully specialized in lentiviral vectors with two areas of expertise:

GMP CDMO: VIVEbiotech is GMP Contract Development and Manufacturing Organization focused on the manufacture of lentiviral vectors from early stages to GMP. Working with European and US-based companies, our main aim is to adapt to your own requirements from a technical and timeline perspective.

Innovation: VIVEbiotech´s technology development strategy is focused on providing a response to the main hurdles that currently exist within the gene therapy field:

- The need for cost-effective processes.
- The enhancement of the safety profile of viral vectors.

VIVEbiotech is working on improved producer cell line development, new pseudo-typing strategies and the development of its own worldwide licensed technology: a non-integrative episomal stable lentiviral vector (LENTISOMA).
www.vivebiotech.com

EXHIBITORS

EXHIBITORS

Booth 47: Xendo is a leading consultancy and project management organisation in the fields of (bio)pharmaceutical products, medical devices and healthcare. We bring our palette of services to companies, ranging from start-ups to multi-national organizations, to provide them with robust solutions. Whether they are a (bio)pharmaceutical or medical device company, a hospital or a pharmacy, a manufacturer or a laboratory, we match their colour. For over 25 years, we have successfully completed thousands of national and international assignments for start-ups as well as for the largest, established multinational companies and organisations. Over 220 experienced and highly educated professionals offer their expertise ranging from strategic advice and project management to auditing, operational support and training; providing a full-colour spectrum.
www.xendo.com

Booth 49: Yposkesi
See page 58
www.yposkesi.com

EDUCATION SESSION

TUESDAY 16 OCTOBER

AFMTÉLÉTHON
CURE THROUGH INNOVATION

09:15-09:30
GARDEN FLOOR: CONFERENCE ROOM 2/3

Organiser and Chair:
Hildegard Büning *Hannover Medical School*

EDUC 1: OPENING WORDS

INV013: Hildegard Büning *Hannover Medical School*
Gene and cell therapy - a brief update

09:30-11:00
GARDEN FLOOR: CONFERENCE ROOM 2/3

EDUC 2: TECH. SPEED DATING

(15 minutes each and 15 for questions)

INV014: Els Verhoeyen
CIRI, INSERM U1111 Lyon, C3M, INSERM U1065, Nice
Novel lentiviral pseudotypes for natural killer based cancer immunotherapies and 'nanoblades' for efficient gene editing in T, B, iPS cells and blood stem cells

INV015: Karim Benihoud
CNRS UMR 8203, University Paris-Sud, Villejuif

INV013: Hildegard Büning
Hannover Medical School
Insights into adenovirus and AAV vectorology

INV016: Eduard Ayuso
INSERM UMR1089, University of Nantes
Manufacturing and quality control of viral vectors

INV017: Claudio Mussolino
University of Freiburg
How to re-write or epigenetic control our genetic information

INV018: Michel Pucéat
INSERM U1251, Marseille
A brief overview on iPSC and embryonic stem cell technology

11:00-11:30
CAMPUS FLOOR

Coffee break

EDUCATION SESSION

TUESDAY 16 OCTOBER

11:30-12:30
GARDEN FLOOR: CONFERENCE ROOM 2/3

EDUC 3: CANCER – WHERE DO WE STAND AND HOW TO MOVE FORWARD?

IINV019: Vincenzo Cerullo *University of Helsinki*
Dressing viruses in tumor's clothing: welcome to the cloning-free oncolytic vaccine era

INV021: Delphine Fessart *INSERM U913, University of Bordeaux*
Cancer stem cells and organoids development: towards a better understanding of the biology behind organoids

12:30-13:15
CAMPUS FLOOR

Lunch

13:15-14:45

EDUC 4: CNS SESSION

INV021: Nicole Déglon *Lausanne University Hospital*
Introduction to gene and cell therapy in the CNS

INV022: Nathalie Cartier *INSERM/ CEA UMR1169, MIRCEN CEA and University Paris-Sud, University Paris Saclay*
Gene therapy for Huntington's disease

INV023: Ron Crystal *Weill Cornell Medical College, New York City, NY*
Gene therapy for Alzheimer's disease

14:45-16:45

EDUC 5: HSC STEM CELLS - WHERE DO WE STAND AND HOW TO MOVE FORWARD

INV025: Adrian Thrasher *University College London Institute of Child Health*
Primary immunodeficiency, gene therapy vs. alternative strategies how do we move into the "real" clinical reality

INV026: Juan Bueren *CIEMAT/CIBERER-ISCIII, Madrid*
Gene therapy in bone marrow failure syndromes

INV027: Olivier Nègre *BlueBirdBio, Cambridge, MA*
Haematopoietic stem cell gene therapy

INV020: Luigi Naldini *SR-TIGET, Milan*
HSC gene therapy: from lentiviral gene transfer to gene editing

16:45-17:00
CAMPUS FLOOR

Coffee break

CLINICAL TRIAL AND COMMERCIALISATION WORKSHOP

TUESDAY 16 OCTOBER

Organisers:
Alessandro Aiuti *SR-TIGET, Milan*
Robin Ali *UCL, Institute of Opthalmology, London*

9:30-11:15
CLOUD FLOOR: AUDITORIUM C

PLANNING AND RUNNING A CLINICAL TRIAL

INV001: Kim Champion
University College London Clinical Trials Centre
General considerations for setting up a clinical trial

INV002: Paola Albertini
SR-TIGET, Milan
Quality requirements for GLP tox testing and GCLP clinical testing

INV003: Marco Anelli
ProductLife Group, Milan
Pharmacovigilance from a sponsor, CRO point of view

INV004: Chiara Bonini
SR-TIGET, Milan
Registry platform for gene and cell therapy: the EBMT approach

11:15-11:45
CAMPUS FLOOR

Coffee

11:45-13:15
CLOUD FLOOR: AUDITORIUM C

MANUFACTURING OF GENE AND CELL PRODUCTS

INV005: Jean-François Brunet
Lausanne University Hospital
Building academic GMP facility

INV006: Xin Swanson
Lonza, Houston, TX Scaling GMP AAV production

INV007: James Miskin
Oxford Biomedica
Scaling GMP lentiviral vector production

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CLINICAL TRIAL AND COMMERCIALISATION WORKSHOP

TUESDAY 16 OCTOBER

13:15-14:15

Lunch

14:15-16:30
CLOUD FLOOR: AUDITORIUM C

PATIENT ACCESS, REGULATORY AND REIMBURSEMENT CHALLENGES

INV008: Ron Philip
Spark Therapeutics, Philadelphia, PA
Patient access worldwide

INV009: Ilona Reischl
Federal Office for Safety in Health Care, Vienna
Update on new ATMP guidelines for GMP production: focus on the risk based approach

INV010: Martina Schüssler-Lenz
CAT Chair, Paul Ehrlich Institute, Langen
Risk management planning in the development of ATMP

INV011: Michela Gabaldo
Telethon, Milan
Market access for ATMP in Europe

INV012: Eric Auger
Putnam Associates, Boston, MA
Market access for ATMP in US

16:30-17:00
CAMPUS FLOOR

Coffee

PROGRAMME

TUESDAY 16 OCTOBER

17:00-19:00

CAMPUS FLOOR: AUDITORIUM A

2018 OPENING SESSION

Chairs:
Robin Ali, Pierre Cordelier, Nicole Deglon, Nancy Witty

INV028: Grégoire Courtine
EPFL, Lausanne
Targeted neurotechnologies enabling walking after paralysis

INV029: Didier Trono (2018 Outstanding Achievement Award lecture)
EPFL, Lausanne
Retroelements, their polydactyl controllers and the specificity of human biology

19:00-20:00

CAMPUS FLOOR

WELCOME RECEPTION

WEDNESDAY 17 OCTOBER

09:00-10:40

GARDEN FLOOR: CONFERENCE ROOM 2/3

S1A: DISEASE MODELLING

Chair:
Amy Wagers

INV030: Eva Hedlund
Karolinska Institute, Stockholm
Elucidating early disease mechanisms in ALS using stem cells

INV031: Holger Willenbring
University California San Francisco, CA
A mouse model of a human cholestatic liver disease reveals extent and therapeutic potential of mammalian transdifferentiation

PROGRAMME

WEDNESDAY 17 OCTOBER

Proffered papers:

OR001: Jamal Alzubi
Institute for Transfusion Medicine and Gene Therapy, Freiburg
A novel chimeric antigen receptor (CAR) T cell approach eliminates prostate cancer in a mouse tumor model

OR002: Saskia König
Institute for Transfusion Medicine and Gene Therapy, Freiburg
Allele-specific editing of STAT3 mutations with CRISPR/Cas in primary cells of hyper-IgE syndrome patients

Presented posters:

P166: Kathrin Haake
Hannover Medical School
Hematopoietic stem cell gene therapy for Ifnyr1 deficiency protects mice from mycobacterial infections and paves way for macrophage transplantation therapy

P167: Viviane Dettmer
Institute for Transfusion Medicine and Gene Therapy, Freiburg
Retroviral UNC13D transfer restores cytotoxic function in T cells derived from familial hemophagocytic lymphohistiocytosis type 3 patients

09:00-10:40

CAMPUS FLOOR: AUDITORIUM A

SESSION 1B: CANCER GENE AND CELL THERAPY

Chairs:
Vincenzo Cerullo, Nicolas Boisgérault

INV032: Waseem Qasim
University College London
Genome engineered T cell immunotherapies for leukaemia

INV033: Kah Whye Peng
Mayo Clinic, Rochester, MN
VSV-IFNb-NIS, an armed and trackable oncolytic vesicular stomatitis virus

PROGRAMME

WEDNESDAY 17 OCTOBER

09:00-10:40

CAMPUS FLOOR:
AUDITORIUM A

Proffered papers:

OR003: Micaela Harrasser
University College London
Effective targeting of ROR1+ solid tumours with next-generation chimeric antigen receptor therapy

Travel grant awarded by:

OR004: Catia Traversari
MolMed S.p.A., Milan
In vivo antitumour activity of a hCD44v6-specific chimeric antigen receptor in syngeneic models of solid tumours

Presented posters:

P093: Chin Yan Chang
Osaka University
RIG-I pathway stimulation polarizes neutrophils to anti-tumor type neutrophil and suppress tumor growth

P019: Silke Uhrig-Schmidt
University Hospital Heidelberg
CARAAVs - a novel class of CAR-antagonists in cancer immunotherapy

P328: Razieh Monjezi
University Hospital Würzburg
CRISPR/Cas9 unites with Sleeping Beauty to generate CAR-T cells with enhanced therapeutic index for fighting against immunosuppressive tumour microenvironment

Travel grant awarded by:

09:00-10:40

CLOUD FLOOR:
AUDITORUM C

SESSION 1C: CNS AND EYE DISEASES GENE AND CELL THERAPY I

ADVERUM BIOTECHNOLOGIES

Chairs: Alberto Auricchio, Yvan Arsenijevic

INV034: Ian MacDonald
University of Alberta, Edmonton, AB
Gene therapy for choroideremia: what have we learned from the clinical trials to date

INV088: Alberto Auricchio
TIGEM, Naples
Expanding AAV transfer capacity in the retina

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PROGRAMME

WEDNESDAY 17 OCTOBER

Proffered papers:

OR005: Samiah Al Zaidy
Centre for Gene Therapy, Nationwide Children's Hospital, Columbus, OH
AVXS-101 phase 1 gene replacement therapy clinical trial in spinal muscular atrophy type 1 (SMA1): 24-month event-free survival and achievement of developmental milestones

OR006: Stylianos Michalakis
Ludwig Maximilian University, Munich
Gene supplementation therapy for CNGA3-linked achromatospia

Presented posters:

P222: Rui Nobre
University of Coimbra
Non-invasive allele-specific silencing therapy and biomarkers for Machado-Joseph Disease

P223: Ruslan Grishanin
Adverum Biotechnologies, Menlo Park, CA
Long-term aflibercept expression levels in non-human primates following intravitreal administration of ADVM-022, a potential gene therapy for wet agerelated macular degeneration

10:40-11:10

Coffee break

11:10-13:10

CAMPUS FLOOR:
AUDITORIUM A

PLENARY SESSION 2: FROM BENCH TO BEDSIDE I

Lonza Pharma & Biotech

Chair: Luigi Naldini

INV036: Stuart Forbes
Edinburgh University, Centre of regenerative medicine
Macrophage therapy for liver disease- preclinical and clinical

INV037: Amy Wagers
Harvard University, Cambridge, MA
In vivo gene editing in tissues and tissue stem cells

PROGRAMME

WEDNESDAY 17 OCTOBER

11:10-13:10
CAMPUS FLOOR: AUDITORIUM A

INV038: Bev Davidson
The Children's Hospital of Philadelphia and The University of Pennsylvania
Emerging therapies for neurodegenerative diseases

INV039: Wing Yen Wong
BioMarin, Novato, CA
Gene therapy in haemophilia: from vision to reality

13:10-15:10
GARDEN & CAMPUS FLOORS

Lunch

POSTER SESSION I : ODD NUMBERS (see p116)
GARDEN FLOOR

13:10-13.30
SFTCG AGM
CAMPUS FLOOR: AUDITORIUM A

13:45-14.45
REGULATORY WORKSHOP
GARDEN FLOOR: CONFERENCE ROOM 2/3
Alison Armstrong

15:10-16:50
GARDEN FLOOR: CONFERENCE ROOM 2/3

SESSION 2A: GENOMIC CHARACTERISATION OF PLURIPOTENT STEM CELLS
Chair: Ludovic Vallier

INV040: Fiona Watt
King's College, London
Identifying extrinsic and intrinsic drivers of variation in cell behaviour in human iPS cell lines

INV041: Kevin Eggan
Harvard University, Cambridge, MA
Reducing noise and bias from studies of disease-implicated genetic variation through massively-mosaic stem cell systems

INV091: Tenneille Ludwig
WiCell, Madison WI
Identification of recurrent genetic variants in hPSCs; a changing landscape

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PROGRAMME

WEDNESDAY 17 OCTOBER

15:10-16:50
CAMPUS FLOOR: AUDITORIUM A

SESSION 2B: VECTOR DEVELOPMENT I
Chairs:
Els Verhoeyen, Axel Schambach

INV043: Jay Chiorini
MPTB, NIDCR, NIH, Bethesda, MD
Characterisation of AAV44.9

INV044: Dirk Grimm
Bioquant, Heidelberg
Small but increasingly mighty - latest advances in AAV biology and vector optimization

Proffered papers:

Travel grant awarded by:

OR007: Kleopatra Rapti
Heidelberg University Hospital
Generation of novel immune-evading AAVs through identification and mutation of immunogenic epitopes in the variable capsid regions of adeno-associated virus 9

OR008: Monica Volpin
SR-TIGET, Milan
The impact of vector integration on chromatin architecture

Presented posters:

Travel grant awarded by:

P483: Weiheng Su
University of Oxford
Exploiting adenovirus mechanisms for the enhanced production of AAV vectors

P484: Jihad El Andari
University Hospital Heidelberg
Identification of new muscle-tropic adeno-associated virus (AAV) capsids for treatment of rare hereditary muscular disorders

PROGRAMME

WEDNESDAY 17 OCTOBER

15:10-16:50

CLOUD FLOOR:
AUDITORIUM C

SESSION 2C: EMA/CAT REGULATORY ASPECTS OF ADVANCED THERAPY MEDICINAL PRODUCTS (ATMPS)

Chairs:
Martina Schüssler-Lenz, Ilona Reischl

INV045: Martina Schüssler-Lenz
CAT Chair, Paul Ehrlich Institute, Langen
Introduction to the committee for advanced therapies (CAT) and its tasks in the evaluation of advanced therapies

INV046: Marcos Timón
Medicines Agency AEMPS, Madrid
EU regulatory aspects of CAR-T cells

INV047: Hans Ovelgönne
Medicines Agency MEB, Utrecht
EU regulatory aspects of rAAV vectors

INV048: Matthias Renner
Paul Ehrlich Institute, Langen
EU regulatory aspects of genome editing

ROUND TABLE DISCUSSION

16:50-17:20

Coffee break

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COLLABORATIVE CONGRESS 2018

PROGRAMME

WEDNESDAY 17 OCTOBER

17:20-18:50

CAMPUS FLOOR:
AUDITORIUM A

PLENARY SESSION 3: CANCER IMMUNOTHERAPY AND CANCER STEM CELLS

Chairs:
Chiara Bonini, Robert Blelloch

INV049: Hinrich Abken
University Hospital Regensburg
CARs and TRUCKs: next generation adoptive cell therapy

INV050: Yasuhiro Yamada
University of Tokyo
Dissecting cancer biology with iPS cell technology

INV051: Robert Blelloch
University of California, San Francisco, CA
Exosomal PD-L1 as an immune-modulator in cancer

20:00-20:30

INVITED SPEAKER DINNER (INVITATION ONLY)

Coaches will leave SwissTech at 19.00 back to the speaker dinner venue

PROGRAMME

THURSDAY 18 OCTOBER

9:00-10:40

CLOUD FLOOR:
AUDITORIUM C

SESSION 3A: BIOENGINEERING

Chair:
Molly Stevens

INV052: David Schaffer
University of California, Berkeley, CA
Molecular elucidation and engineering of stem cell fate decisions

INV053: Penney Gilbert
University of Toronto, ON
Making 3D models that matter: engineering skeletal muscle tissue in a dish

Proffered papers:

OR009: Pauline Schmit
Harvard University, Cambridge, MA
Cross-packaging control in multiplexed AAV libraries

OR010: François du Plessis
uniQure, Amsterdam
Development of a next generation synthetic promoter for liver directed gene therapy

Presented posters:

P168: Ekaterina Naumenko
Kazan Federal University
Enchanced dark-field microscopy for histological detection of nanostructured scaffolds after implantation into bone defects

P169: Albert Rizvanov
Kazan Federal University
Migration ability of human polymorphonuclear leukocytes loaded with synthetic microcapsules

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PROGRAMME

THURSDAY 18 OCTOBER

9:00-10:40

GARDEN FLOOR:
CONFERENCE
ROOM 2/3

SESSION 3B: MUSCLE & CARDIOVASCULAR DISEASES

Chairs:
Michel Pucéat, Uta Griesenbach

INV054: Ana Buj Bello
Genethon, Evry
Gene therapy of myotubular myopathy: from preclinical studies to a clinical trial

INV055: Silvia Priori
Istituti Clinici Scientifici Maugeri, Pavia
Gene therapy to prevent chaotic behaviours in cardiac electrophysiology

INV056: Antoine de Vries
Leiden University Medical Centre
Shining light on cardiac tachyarrhythmias

Presented posters:

P392: Capucine Trollet
Sorbonne University, Paris
BB-301: a single "silence and replace" AAV-based vector for the treatment of oculopharyngeal muscular dystrophy (OPMD)

P247: Francesco Tedesco
University College London
High-fidelity disease modelling of skeletal muscle laminopathies using LMNA-mutant human iPS cells and bioengineered muscles

PROGRAMME

THURSDAY 18 OCTOBER

9:00-10:40

CAMPUS FLOOR:
AUDITORIUM A

SESSION 3C: BLOOD DISORDERS I

Chairs:
Juan Bueren, Waseem Qasim

INV057: Marina Cavazzana
Imagine Institute, Paris
Gene therapy of hemoglobinopathies

INV058: Bernhard Gentner
SR-TIGET, Milan
Towards next-generation gene therapy with *ex vivo*-engineered haematopoietic stem and progenitor cells

Proffered papers:

OR011: Frank Staal
Leiden University Medical Centre
Developing stem cell-based gene therapy for RAG1 deficient-SCID

OR012: Paula Rio
CIEMAT-CIBERER-IIS/FJD, Madrid
Gene therapy trial in non-conditioned Fanconi anemia patients

Presented posters:

P052: Carlos Carrascoso
CIEMAT-CIBERER-IIS/FJD, Madrid
Towards the gene therapy of the bone marrow failure in patients with dyskeratosis congenita

P053: Pamela Quaranta
SR-TIGET, Milan
Role of peripheral blood circulating haematopoietic stem/progenitor cells during physiological hematopoietic maturation and after gene therapy

Travel grant awarded by:

10:40-11:10 **Coffee break**

PROGRAMME

THURSDAY 18 OCTOBER

11:10-12:40

CAMPUS FLOOR:
AUDITORIUM A

PLENARY SESSION 4: FROM BENCH TO BEDSIDE II

Chairs: Adrian Thrasher, Malin Parmar

INV059: Deepak Srivastava
Gladstone Institutes, San Francisco, CA
Cardiac development: basis for disease and regeneration

INV060: Kristin Baldwin
Scripps Research, San Diego, CA
Precision reprogramming approaches to cardiovascular and neurologic disease

INV061: Lorenz Studer
Memorial Sloan Kettering Cancer Center, New York, NY
Towards a pluripotent-based cell therapy for Parkinson's disease

12:40-14:40 **Lunch**

POSTER SESSION II : EVEN NUMBERS (see p138)

GARDEN FLOOR

13.15-14.15

WORKSHOP ON THE USE OF LENTIVIRAL VECTORS FOR IN VIVO GENE THERAPY

GARDEN FLOOR: CONFERENCE ROOM 2/3

Speakers: Luigi Naldini, *SR-TIGET, Milan*
Liver-directed lentivral gene therapy of hemophilia
Deborah Gill, *University of Oxford*
Lung-targeted SIV gene therapy for cystic Fibrosis
Kyri Mitrophanous, *Oxford Biomedica*
Clinical experience of local administration of lentiviral vectors in retinal disorders and Parkinson's disease

PROGRAMME

THURSDAY 18 OCTOBER

14:40-16:50

GARDEN FLOOR: CONFERENCE ROOM 2/3

SESSION 4A: MOLECULAR BASIS OF DEVELOPMENTAL POTENTIAL

Chair: Amander Clark

INV062: Jennifer Erwin
The Lieber Institute for Brain Development, Baltimore, MD
Repetitive elements in stem cells

INV063: Ludovic Vallier
Wellcome Sanger Institute, Cambridge
Mechanisms controlling cell fate decisions in human pluripotent stem cells

Proffered papers:

OR026: Alberto De Iaco
EPFL, Lausanne
DPPA2 and DPPA4 regulate expression of Dux in mouse embryonic stem cells

14:40-16:50

CLOUD FLOOR: AUDITORIUM C

SESSION 4B: VECTOR DEVELOPMENT II

Chairs: Hildegard Büning, Karim Benihoud

INV064: Rob Kotin
University of Massachusetts Medical School, Worcester, MA
Orthologous dependoparvovirus molecular fossils may provide a source of novel structural motifs and capsids for rAAV

INV065: Jude Samulski
University of North Carolina, Chapel Hill, NC
Gene therapy for DMD: from bench to bedside

INV066: Eduard Ayuso, *University of Nantes*
Starting from the end: analytics driving the manufacturing process of viral vectors

Proffered papers:

OR014: Mathieu Mevel, *University of Nantes*
NextGenAAV: a mix of organic chemistry and vectorology
OR015: Sonja Kleinlogel, *University of Bern*
Evolution of recombinant adeno-associated viral vectors for favorable retinal penetration properties

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PROGRAMME

THURSDAY 18 OCTOBER

Presented posters:
P485: Pasqualine Colella
University of Paris-Saclay
Tandem promoter design confers tolerogenic and persistent transgene expression to AAV gene therapy in neonate Pompe mice

14:40-16:50

CAMPUS FLOOR: AUDITORIUM A

SESSION 4C: GENE EDITING

Chairs: Keith Joung, Paula Rio

INV067: Toni Cathomen
University of Freiburg
New insights in CRISPR/Cas specificity, DNA repair dynamics and DNA repair outcomes in gene edited human haematopoietic stem cells

INV068: Yong Chang
Intellia Therapeutics, Cambridge, MA
Delivering on the therapeutic potential of CRISPR/Cas9: development of an LNP-mediated genome editing therapeutic for the treatment of ATTR

Proffered papers:
OR016: Gerald Schwank
ETH, Zürich
Correction of autosomal recessive disorders via CRISPR-associated base editors in adult animals
OR017: Jae Young Lee
ToolGen, Seoul
CRISPR/Cas9-mediated downregulation of PMP22 ameliorates Charcot-Marie-Tooth disease 1A in mice
OR018: Jonathan Finn
Intellia Therapeutics, Cambridge, MA
Supra-therapeutic levels of transgene expression achieved *in vivo* by CRISPR/Cas9 mediated targeted gene insertion
OR019: Anais Amaya
The University of Sydney, NSW
Successful *in vivo* editing of patient-derived primary human hepatocytes

Travel grant awarded by:

PROGRAMME

THURSDAY 18 OCTOBER

Presented posters:

P298: Alessia De Caneva
ICGEB, Trieste
Coupling AAV-mediated promoterless gene targeting to SaCas9 nuclease to efficiently correct liver metabolic diseases

P299: Antonio Casini
University of Trento
evoCas9, a highly specific SpCas9 variant from a yeast *in vivo* screening

P300: Els Verhoeyen
CIRI, INSERM U1111 Lyon, C3M, INSERM U1065, Nice
Efficient genome editing in primary human T, B and HSCs using Baboon envelope gp pseudotyped virus derived "Nanoblades" loaded with Cas9/sgRNA ribonucleoproteins

Travel grant awarded by:

16:50-17:20 **Coffee break**

17:20-19:20

CAMPUS FLOOR:
AUDITORIUM A

PLENARY SESSION 5: NEW TOOLS AND TECHNOLOGIES

Chairs: Robin Ali, Gordon Keller

INV069: Keith Joung
Harvard Medical School, Cambridge, MA
In vivo CRISPR gene editing with no detectable genome-wide off-target mutations

INV070: David Russell
University of Washington and Universal Cells, Seattle, WA
Universal donor stem cells

INV71: Botond Roska
Friedrich Miescher Institute for Biomedical Research, Basel
The human retina and its organoids at single cell resolution

INV072: Alex Meissner
Max Planck Institute for Molecular Genetics, Berlin
Differential regulation of Oct4 targets facilitates reacquisition of pluripotency

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PROGRAMME

THURSDAY 18 OCTOBER

20:00

MOLECULAR MINGLE AT THE OLYMPIC MUSEUM

Coaches will leave from SwissTech at 19.30 to the Olympic Museum

FRIDAY 19 OCTOBER

09:00-10:40

CLOUD FLOOR:
AUDITORIUM C

SESSION 5A: CELL THERAPY AND REPLACEMENT

Chair:
Stuart Forbes

INV073: Asuka Morizane
University of Kyoto
Cell therapy for Parkinson's disease with induced pluripotent stem cells

INV074: Hanna Mikkola
University of California, Los Angeles, CA
MLLT3 governs human hematopoietic stem cell self-renewal

Proffered papers:

OR020: Andrea Schejtman
University College London
Towards clinical application of a lentiviral gene therapy protocol for p47phox deficient chronic granulomatous disease

OR021: Ilaria Meloni
University of Siena
Toward gene editing in Rett syndrome

Presented posters:

P170: Immacolata Brigida
SR-TIGET, Milan
Gene therapy for adenosine deaminase 2 deficiency

PROGRAMME

FRIDAY 19 OCTOBER

09:00-10:40

CAMPUS FLOOR: AUDITORIUM A

SESSION 5B: METABOLIC AND LYSOSOMAL DISEASES

Spark Therapeutics

Chairs:
Federico Mingozzi, Fatima Bosch

INV075: Juan Ruiz
Abeona Therapeutics, Dallas, TX
Treatment of lysosomal storage diseases (MPS-IIIA and IIIB) by intravenous administration of AAV vectors

INV076: Michel Zerah
Necker Hospital, Paris
Intracerebral gene therapy: neurosurgical point of view

Proffered papers:

OR023: Brian Bigger
University of Manchester
Brain targeted stem cell gene therapy corrects mucopolysaccharidosis type II via multiple mechanisms?

OR022: Joseph Lillegard
Mayo Clinic, Rochester, MN
In utero liver-directed lentiviral gene therapy cures a pig model of hereditary tyrosinemia type 1

Presented posters:

P357: Sem Aronson
University of Amsterdam
Brain targeted stem cell gene therapy corrects Mucopolysaccharidosis type II via multiple mechanisms

Travel grant awarded by:
EUROPEAN SOCIETY OF GENE & CELL THERAPY

ROUND TABLE

PROGRAMME

FRIDAY 19 OCTOBER

09:00-10:40

GARDEN FLOOR: CONFERENCE ROOM 2/3

SESSION 5C: BLOOD DISORDERS II

Chairs:
Olivier Nègre, Thierry VandenDriessche

INV077: Isabelle André-Schmutz
Institute Imagine, Paris
Ex vivo generated lymphoid progenitors for immune reconstitution in the context of allogeneic transplantation and gene therapy

INV078: Basil Sharrack
University of Sheffield
Autologous hematopoietic cell transplantation in multiple sclerosis

Proffered papers:

OR024: Elena Almarza Novoa
CIEMAT, Madrid
Comprehensive preclinical studies for the gene therapy of patients with leukocyte adhesion deficiency type I (LAD-I)

OR025: Ilana Moscatelli
Lund University
Haematopoietic stem cell targeted neonatal gene therapy by a clinically applicable lentiviral vector corrects osteopetrosis in oc/oc mice

Presented posters:

P054: Elena Barbon
University of Evry
The potential use of nanobodies delivered via AAV vectors in the treatment of haemophilia

P171: Cristina Olgassi
University of Eastern of Piedmont Orientale, Novara
Patient-specific iPSC-derived endothelial cells provide long-term phenotypic correction of haemophilia A

10:40-11:10

Coffee break

PROGRAMME

FRIDAY 19 OCTOBER

11:10-13:10

CAMPUS FLOOR: AUDITORIUM A

PLENARY SESSION 6: ORGANOIDS AND CELL ENGINEERING

Chairs: Robin Ali, Deepak Srivistava

INV079: Molly Stevens
Imperial College London
Exploring and engineering the cell-material interface for regenerative medicine and mechanobiology

INV080: Rick Livesey
University of Cambridge
Engineering stem cell-derived human neural tissues to study brain development and disease

INV081: Hiromitsu Nakauchi
University of Tokyo
From cells to organs: exploiting the organ niche for interspecies organogenesis

INV082: Gordon Keller
University of Toronto
Translating human development to new therapies with pluripotent stem cells

13:10-15:00

Lunch

POSTER SESSION III : ALL POSTERS

GARDEN FLOOR

13.45-14.45

EARLY CAREER RESEARCHERS CAREER'S WORKSHOP

GARDEN FLOOR: CONFERENCE ROOM 2/3

Vincenzo Cerullo, *University of Helsinki*
Deniz Kirik, *Lund University*

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FRIDAY 19 OCTOBER

13.45-14.45

GENE THERAPY FOR THE 21ST CENTURY PATIENT

CLOUD FLOOR AUDITORIUM

Chairs:
Nathalie Cartier, *MIRCen, INSERM U986, CEA, Fontenay aux Roses*
Samantha Parker, *Lysogene, Paris*

Speakers:
Annie Hubert, *Alliance for Regenerative Medicine (ARM)*
Nicole Boice, *Global Genes*
Cara O'Neill, *Cure Sanfilippo Foundation*
Elin Haf Davies, *Aparito*

15:00-16:30

GARDEN FLOOR: CONFERENCE ROOM 2/3

SESSION 6A: REGULATION AND CONTROL OF PURIPOTENCY AND LINEAGE SPECIFICATION?

Chair: Teneille Ludwig

INV083: Keisuke Kaji
Edinburgh University
Molecular mechanisms of cellular reprogramming

INV084: Amander Clark
University of California Los Angeles, CA
Unique control of naïve pluripotency in human stem cells and the germline

Proffered papers:

OR013: Julien Pontis
EPFL, Lausanne
Evolutionarily recent transposable elements and their controllers regulate human early embryonic transcriptional network

Presented posters:

P371: Pavel Makarevich
Moscow State University
Role of paracrine factors secreted by mesenchymal stromal cells in spermatogonial stem cell niche regulation

PROGRAMME

FRIDAY 19 OCTOBER

15:00-16:30

CLOUD FLOOR:
AUDITORIUM C

SESSION 6B: IMMUNE RESPONSES FOLLOWING GENE THERAPY: FROM VACCINES TO VECTOR OPTIMISATION

Chairs: Anne Galy, Jude Samulski

INV085: Maria Croyle
University of Texas, Austin
Reaching beyond the cold chain: formulation design of vaccines to improve potency, enhance distribution and modulate other biological processes

INV086: Ying Kai Chan
Harvard University, Cambridge, MA
Engineering AAV vectors to evade innate immune and inflammatory responses

Proffered papers:

OR027: Dimitrios Laurin Wagner
Charité, Berlin
T cell immunity towards CRISPR-associated nucleases

OR028: Tim Beissert
TRON, Mainz
Alphaviral trans-replicating RNA is a low dose vaccine vector

15:00-16:30

CAMPUS FLOOR:
AUDITORIUM A

SESSION 6C: CNS AND EYE DISEASES II

brammer bio

Chairs:
Nathalie Cartier, Nicole Deglon

INV087: Shin-Ichi Muramatsu
Jichi Medical University
Gene therapy for Parkinson's disease, implications from a clinical study of AADC deficiency

INV035: Sandro Alves
Brainvectis, Paris
CYP46A1-gene therapy alleviates spinocerebellar ataxia in mouse models

PROGRAMME

FRIDAY 19 OCTOBER

REGENXBIO

Proffered papers:

OR029: Vania Broccoli
San Raffaele Hospital, Milan
Modeling functional and dysfunctional brain circuits with human iPSC-derived neurons in microfluidic chambers

OR030: Lee Ni-Chung
National Taiwan University Hospital, Taipei
Gene therapy for AADC deficiency results in de novo dopamine production and supports durable improvement in major motor milestones

16:30-17:00

Coffee break

17:00-19:00

CAMPUS FLOOR:
AUDITORIUM A

AUDENTES

PRESIDENTIAL SYMPOSIUM
ESGCT AGM

Chairs:
Robin Ali, Pierre Cordelier, Nicole Deglon, Nancy Witty

INV089: Juergen Knoblich
Institute of Molecular Biotechnology, Vienna
Cerebral organoids: modelling human brain development and tumorigenesis in stem cell derived 3D culture

INV090: Rudolf Jaenisch
MIT, Cambridge, MA
Epigenetic regulation in development, ageing and disease

Proffered papers:

OR031: Alessio Cantore (Young Investigator Award lecture)
SR-TIGET, Milan
Shielding lentiviral vectors from phagocytosis increases hepatocyte gene transfer in non-human primates

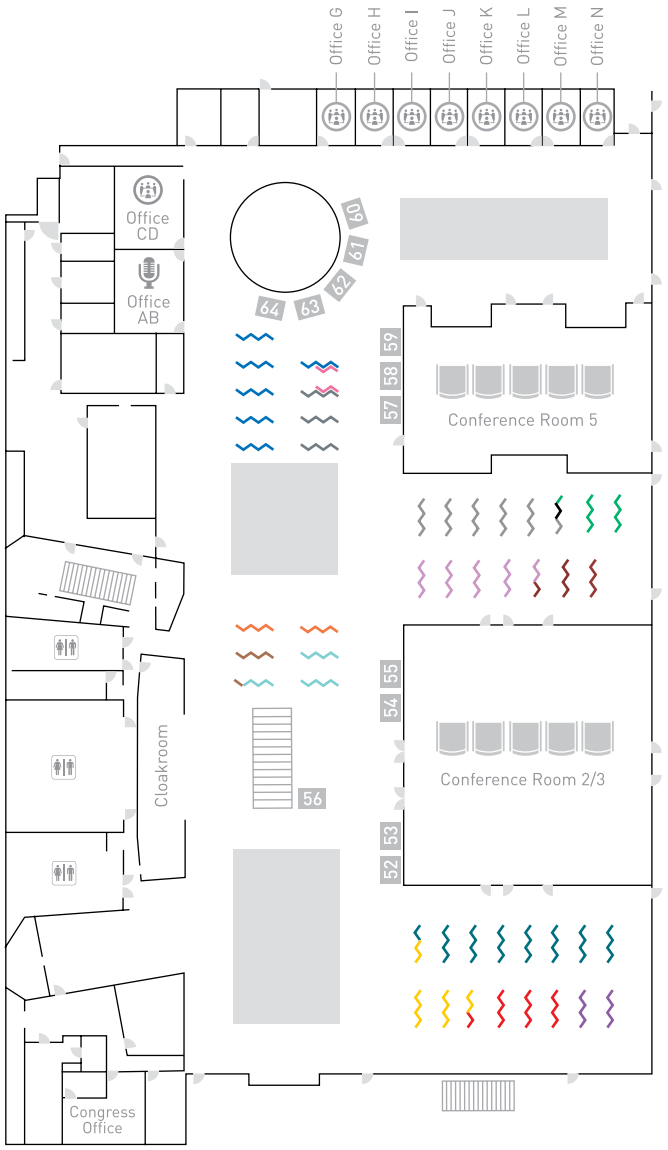
19:00-19:30

CLOSING DRINKS

POSTER SESSIONS

- **P001–P018** Bioengineering
- **P023–p054** Blood disorders
- **P060–P093** Cancer
- **P100–P172** Cell therapy and replacement
- **P177–P223** CNS and ocular diseases
- **P226–P247** Disease modelling
- **P250–P302** Gene editing
- **P305–P306** Genomic characterisation of pluripotent stem cells
- **P307–P328** Immunotherapy and genetic vaccines
- **P330–P357** Metabolic and lysosomal diseases
- **P360–P371** Molecular basis of development potential
- **P373–P392** Muscle, cardiovascular and pulmonary diseases
- **P395–P422** Other
- **P425–P430** Regulation and control of puripotency and lineage specification
- **P435–P492** Vector develpment

GARDEN FLOOR:



POSTER SESSION 1 / 17 OCT

GARDEN FLOOR
13.10 - 15.10

BIOENGINEERING

P001

Philippe Véron *Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*

Development of an AAV-specific plasmapheresis device for the selective removal of anti-capsid antibodies

P003

Mohamed Zoughaib *Kazan Federal University*

Interaction of implanted zinc-containing cryogel with tissue environment

P005

Jean-Francois Brunet *Lausanne University Hospital*

Cell manufacturing facility at Lausanne university hospital: large experience of autologous skin cells for burns patients

P007

Tomas Kostelec *BIA Separations, Ajdovščina*

HEK-derived AAV purification: comparison of small scale laboratory production towards industrial format using monoliths

P009

Jacek Lubelski *uniQure, Amsterdam*

rAAV large scale manufacturing using BEVS technology

P011

Wioletta Lech *Mossakowski Medical Research Centre, Polish Academy of Sciences*

Molecular evaluation of neuroprotective properties of WJ-MSC in different microenvironmental conditions

BIOENGINEERING

P013

Ekaterina Naumenko *Kazan Federal University*

Identification of nano particle mixtures in human cell culture using dark-field and hyperspectral imaging

P015

Ekaterina Naumenko *Kazan Federal University*

Silver-infused halloysite nanotubes as an antibacterial nanocomposite in cell therapy

P017

Albert Rizvanov *Kazan Federal University*

Effect of titanium nickelide reticular membranes on mesenchymal stem cells *in vitro*

P019

Silke Uhrig-Schmidt *NCT University Hospital Heidelberg*

CARAAVs - a novel class of CAR-antagonists in cancer immunotherapy

BLOOD DISORDERS

P023

Katelyn Masiuk *Department of Microbiology, Immunology and Molecular Genetics, University of California, Los Angeles, CA*

dmPGE2 and poloxamer-F108 enhance transduction of human hematopoietic stem and progenitor cells with a ß-globin lentiviral vector

P025

Riccardo Biavasco *SR-TIGET, Milan*

Oncogene-induced senescence in haematopoietic progenitors features myeloid-restricted hematopoiesis and histiocytosis

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BLOOD DISORDERS

P027

Yari Gimenez Martinez *CIEMAT/ CIBERER-ISCIII, Madrid*

Preclinical studies towards the gene therapy of Diamond-Blackfan anemia

P029

Els Verhoeyen *CIRI, INSERM U1111 Lyon, C3M, INSERM U1065, Nice*

BaEV pseudotyped LVs confer FVIII gene transfer in HSCs, allowing secretion of functional factor FVIII from a B-cell specific promoter *in vivo* in NSG FVIII $^{-/-}$ mice

P031

Oscar Quintana Bustamante *CIEMAT/ CIBERER-ISCIII, Madrid*

Preclinical biosafety studies of lentiviral vector-mediated gene therapy in erythrocyte pyruvate kinase deficiency

P033

Cristina Mesa *CIEMAT/CIBERER-ISCIII, Madrid*

Efficient and cost effective transduction of hematopoietic stem cells with lentiviral vectors for the treatment of leukocyte adhesion deficiency type I

P035

Eleanor Luce *Inserm UMR 1193, Villejuif*

Proof of concept for autologous cell/gene therapy of hemophilia B using patient's specific iPSC-derived hepatocytes after genetic correction with CRISPR/Cas9 technology

BLOOD DISORDERS

P037

Kavitha Rajavel *Shire, Dublin*

Safety and dose escalation of BAX 888 (SHP654), an AAV8 vector expressing B-domain deleted factor VIII in patients with severe haemophilia A: design of a global, open-label, multicentre Phase 1/2 study

P039

Laura Garcia Perez *Leiden University Medical Center*

Developing SIN lentiviral vectors to correct RAG2 deficiency

P041

Daniela Cesana *SR-TIGET, Milan*

Addressing the impact of vector genotoxicity on the dynamics of hematopoietic reconstitution by integration site analyses

P043

Carlos Carrascoso-Rubio *CIEMAT/ CIBERER-ISCIII, Madrid*

The engraftment of lentivirally transduced hCD34^{+} cells in non-conditioned NSG mice is not altered due to a mobilization regimen with G-CSF/AMD3100

P045

Juan A. Hernandez Bort *Shire, Gene Therapy Center Austria, Vienna*

Lyophilisation cycle development of AAV gene therapy product

P047

Juan A. Hernandez Bort *Shire - Gene Therapy Center Austria, Vienna*

Biological activity of additional AAV subpopulation in AAV gene therapy product

POSTER SESSION 1 / 17 OCT GARDEN FLOOR 13.10 - 15.10

BLOOD DISORDERS

P049

Ning Wu *DKFZ and NCT, Heidelberg*
Exploring the HIV-1 integration sites with different methods

P051

Mariya Tikhomirova *Kazan Federal University*
Effect of dexamethasone on autophagy induction in T-lymphocytes of patients with mild bronchial asthma

P053

Pamela Quaranta *SR-TIGET, Milan*
Role of peripheral blood circulating haematopoietic stem/progenitor cells during physiological hematopoietic maturation and after gene therapy

CANCER

P061

Pierre Cordelier *Inserm U1037, Toulouse*
The antitumoral activity of TLR7 ligands is corrupted by the microenvironment of pancreatic tumors

P063

Philippe Erbs *Transgene, Strasbourg*
Vaccinia virus shuffling: deVV5, a novel chimeric poxvirus with improved oncolytic potency

P065

Olakunle Oladimeji *University of KwaZulu-Natal, Westville*
Laminin receptor dependent gold nanoparticles for mitochondrial targeted delivery in cancer

CANCER

P067

Johann Foloppe *Transgene, Strasbourg*
TG6002: a novel oncolytic and vectorised gene-prodrug therapy approach to target and treat cancer

P069

Pierre Cordelier *Inserm U1037, Toulouse*
Role of components of microRNA machinery in carcinogenesis: targeting DGCR8 impairs pancreatic tumors growth

P071

Tomoyuki Nishikawa *Osaka University*
Development of RNA drug to induce antitumour immunity and cancer selective apoptosis

P073

Mariangela Garofalo *University of Milan*
Extracellular vesicles enhance the targeted delivery of immunogenic oncolytic adenovirus in immunocompetent mice

P075

Shuji Kubo *Hyogo College of Medicine, Nishinomiya*
Human mesenchymal stem cells as cellular vehicles to deliver retroviral replicating vectors for cancer gene therapy

P077

Sunil Arora *PGIMER, Chandigarh*
Breast cancer stem cells (BCSCs) associate with aggressive tumors and cause field cancerization in breast cancer

P079

Zaneta Slyk *Medical University of Warsaw*
Topical administration of rAAV/carboxymethylocellulose formulations – *in vitro* and *in vivo* tests

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POSTER SESSION 1 / 17 OCT GARDEN FLOOR 13.10 - 15.10

CANCER

P081

Albert Rizvanov *Kazan Federal University*
Generation of genetically engineered canine mesenchymal stem cells co-expressing immunomodulating cytokines and tumour suppressors

P083

Albert Rizvanov *Kazan Federal University*
Self-organization and cell proliferation of adipose derived stem cells, HeLa and mononuclear cells after co-culture

P085

Mariya Tikhomirova *Kazan Federal University*
Non-small lung cancer cells exhibit signs of EMT in response to cisplatin treatment and after development of cisplatin resistance

P087

Angelina Titova *Kazan Federal University*
Possibilities of application of photodynamic therapy for the treatment of squamous cell carcinoma of the oesophagus and bronchus

P089

Albert Rizvanov *Kazan Federal University*
IL-2 genetically modified mesenchymal stem cells demonstrate increased VEGF, MMP2 and TGF-ß1 genes expression

P093

Chin yang Chang *Osaka University*
RIG-I pathway stimulation polarizes neutrophils to anti-tumor type neutrophil and suppress tumor growth

CELL THERAPY AND REPLACEMENT

P101

Catarina Miranda *CNC, University of Coimbra*
Repeated mesenchymal stromal cells treatment sustainably alleviates Machado-Joseph disease/spinocerebellar ataxia type-3

P103

Raffaella Di Micco *SR-TIGET, Milan*
Impact of DNA damage response activation in haematopoietic stem and progenitor cells upon gene-targeting

P105

Marianna Paulis *National Research Council (CNR) – IRGB/UOS of Milan*
Chromosome transplantation in human iPS cells as a possible approach to treat disorders due to X chromosome abnormalities

P107

Julia Hanauer *Paul Ehrlich Institute, Langen*
In vivo generation of CAR-T cells by CD4- and CD8-targeted lentiviral vectors

P109

Marine Charrier *INRA/Oniris UMR 703, Nantes*
Demonstration of immunomodulatory properties for human MuStem cell population, a promising candidate for cell therapy of muscular dystrophies

P111

Walter Habeler *I-STEM, Corbeil-Essonnes*
Establishment of a tissue engineered product consisting of RPE derived from clinical grade human embryonic stem cell line cultured on human amniotic membrane for clinical applications

POSTER SESSION 1 / 17 OCT GARDEN FLOOR 13.10 - 15.10

CELL THERAPY AND REPLACEMENT

P113

Alice Rannou *INRA/Oniris UMR 703, Nantes*
Myocardial infarct repair with human adult muscle-derived stem cells "MuStem"

P115

Cristina Olgasi *University of Piemonte Orientale, Novara*
Application of combined gene and cell therapy within an implantable therapeutic device for the treatment of severe haemophilia A

P117

Anna Rafiei *Hannover Medical School*
Generation of human iPSC-derived macrophages using a GMP-compliant process pipeline

P119

Giulia Ferrari *University College London*
Combining iPS cell-derived myogenic progenitors and human artificial chromosomes for genomic-integration-free, systemically deliverable cell and gene therapy of Duchenne muscular dystrophy

P121

Chiara Cugno *Sidra Medicine, Qatar*
Clinical grade production of mesenchymal stromal cells from adipose tissue and foreskin

P123

Martin Wolf *Medical University, Salzburg*
Extracellular vesicles of placental expanded stromal cells have immunomodulatory capacity and pro-angiogenic function

CELL THERAPY AND REPLACEMENT

P125

Antonio Lo Nigro *Hubrecht Institute, Utrecht*
Dissecting pancreatic exocrine heterogeneity to identify a bi-potent progenitor

P127

Luiza de Macedo Abdo *Brazilian National Cancer Institute, Rio de Janeiro*
Chimeric antigen receptor modified T cells using Sleeping Beauty system by electroporation and combination with co-culture expansion has cytotoxic activity *in vitro* and *in vivo*

P129

Nuttha Klincumhom *Chulalongkorn, Bangkok*
The effect of Toll-like receptor 3 (TLR3) on regenerative properties of human periodontal ligament stem cells (PDLSCs)

P131

Liliana Mendonça *University of Coimbra*
Generation and transplantation of patient-specific iPSC-derived neuroepithelial stem cells for Machado-Joseph disease treatment

P133

Chuti Laowtammathron *SiSCR, Mahidol University*
Enhanced generation of human embryonic stem cells with minimized trophoblast cell proliferation for clinical application

P135

Amina Ahmad *The University of Sydney and Sydney Children's Hospitals Network, Westmead, NSW*
Reduced activity of the complement system through elevation of complement factor I using AAV mediated gene delivery to the liver

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CELL THERAPY AND REPLACEMENT

P137

Pavel Makarevich *Lomonosov Moscow State University*
Optimization of mesenchymal stromal cells culture in serum-free media using cell-free extracellular matrix biomaterial

P139

Albert Rizvanov *Kazan Federal University*
Cell mediated gene therapy for neurodegenerative diseases, neurotrauma and stroke

P141

Els Verhoeyen *CIRI, INSERM U1111 Lyon, C3M, INSERM U1065, Nice*
Baboon retrovirus envelope pseudotyped lentivectors permit robust transduction of NK cells and represent and improved tool for cancer immunotherapy

P143

Albert Rizvanov *Kazan Federal University*
Analysis of the interaction of mesenchymal stem cells with artificial human neuroblastoma cell-derived microvesicles using imaging flow cytometry

P145

Pavel Makarevich *Lomonosov Moscow State University*
Development of novel antifibrotic therapy based on human MSC secretome

P147

Ekaterina Naumenko *Kazan Federal University*
Influence of magnetic nanoparticles stabilized with polyelectrolytes on 2D and 3D cell cultures formation

CELL THERAPY AND REPLACEMENT

P149

Ekaterina Naumenko *Kazan Federal University*
"Smart" nanostructured scaffolds for osteoregeneration in veterinary

P151

Maxim Baltin *Kazan Federal University*
Micellar formulation of methylprednisolone succinate for local therapy of spinal cord injury in rats

P153

Yeonmi Lee *University of Ulsan College of Medicine, ASAN Institute for Life Sciences, ASAN Medical Center, Seoul*
Therapeutic potential of iPSCs derived from a patient with mitochondria disease

P155

Albert Rizvanov *RASA Center, Kazan Federal University*
Ultrastructure of human adipose-derived multipotent mesenchymal stromal cells loaded with synthetic microcapsules

P157

Alessandra Castelli *National Research Council (CNR) – IRGB/UOS of Milan*
Chromosome transplantation to correct the chronic granulomatous disease defect in mouse pluripotent stem cells

P159

Artur Fedianin *Kazan Federal University*
Immunoexpression of PSD95 and synaptophysin in the motononeurons of the lumbosacral spinal segments of mouse during experimental hypogravity

POSTER SESSION 1 / 17 OCT GARDEN FLOOR 13.10 - 15.10

CELL THERAPY AND REPLACEMENT

P161

Diane Seimetz *Biopharma Excellence, Munich*
How to find the right dose for successful transition of ATMPs from the nonclinical to the clinical development stage

P163

Da young Chang *Ajou University, Suwon*
Assessment of bystander effects of mesenchymal stem cells carrying a cytosine deaminase suicide gene on glioma cells

P165

Andrew Crane *Department of Neurosurgery, University of Minnesota, Minneapolis, MN*
Non-hematopoietic umbilical cord blood stem cells exert a neuroprotective effect via reduced numbers of infiltrating immune cells in chronic stroke and traumatic brain injury

P167

Viviane Dettmer *University Hospital Freiburg*
Retroviral UNC13D transfer restores cytotoxic function in T cells derived from familial hemophagocytic lymphohistiocytosis type 3 patients

P169

Albert Rizvanov *Kazan Federal University*
Migration ability of human polymorphonuclear leukocytes loaded with synthetic microcapsules

P171

Cristina Olgasi *University of Piemonte Orientale, Novara*
Patient-specific iPSC-derived endothelial cells provide long-term phenotypic correction of hemophilia A

CNS AND OCULAR DISEASES

P177

Rosario Sánchez-Pernaute *Andalusian Initiative for Advanced Therapies, Seville*
Isolation and characterization of CSF-NSCs from premature infants with intraventricular haemorrhage

P179

Serena Giannelli *San Raffaele Scientific Institute, Milan*
A novel AAV design improving efficiency and safety in a gene therapy approach for Rett syndrome in a mouse model

P181

Patrizia Tornabene *TIGEM, Naples*
AAV-mediated protein trans-splicing in the retina

P183

Sonia Duarte *CNC, University of Coimbra*
Let-7 microRNA overexpression mediated by lentivirus alleviates neuropathology, balance and motor deficits in Machado-Joseph disease mouse models

P185

Holger Patzke *Voyager Therapeutics, Cambridge, MA*
Robust SOD1 knockdown in large mammal spinal cord using a novel delivery paradigm with AAV gene therapy targeting SOD1 for the treatment of SOD1-ALS

P187

Astrid Valles Sanchez *uniQure, Amsterdam*
Sustained mutant Huntingtin lowering in the brain and cerebrospinal fluid of Huntington disease minipigs mediated by AAV5-miHTT gene therapy

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CNS AND OCULAR DISEASES

P189

Rajashekhar Gangaraju *University of Tennessee Health Science Center, Memphis, TN*
Adipose stem cells and their paracrine factors are therapeutic for early retinal complications of diabetes in the Ins2Akita mouse

P191

Gabriel Vachey *Lausanne University Hospital*
Allele-specific gene editing for Huntington's disease mediated by a self-inactivating CRISPR/Cas9 system

P193

Gaia Colasante *San Raffael Scientific Institute, Milan*
dCas9-based Scn1a gene activation restores inhibitory interneuron excitability and attenuates seizures in Dravet syndrome mice

P195

Heikki Turunen *AGTC, Cambridge, MA*
Effect of neutralizing anti-AAV antibodies on vector transduction following intravitreal administration of AAV in non-human primates

P197

Liliane Tenenbaum *Lausanne University Hospital*
Long-term uninterrupted high dose treatment reduces the beneficial effects of GDNF gene delivery

P199

Ellie Crompton *Royal Holloway, University of London*
Using integration-deficient lentiviral vectors as a therapeutic strategy for spinal muscular atrophy

CNS AND OCULAR DISEASES

P201

Kyriacos Mitrophanous *Oxford BioMedica*
Prosavin®, a dopamine gene therapy for advanced Parkinson's disease: 5 years phase I/II clinical update

P203

Albert Rizvanov *Kazan Federal University*
Effects of VEGF and FGF2 gene therapy on sciatic nerve regeneration in rats

P205

Thais Bascuas Castillo *University Hospitals of Geneva*
Non-viral transfections with the neuroprotective factors PEDF and GM-CSF reduces oxidative stress in human pigment epithelium cells *in vitro* offering a promising approach to treat avascular age-related macular degeneration

P207

Albert Rizvanov *Kazan Federal University*
Application of autologous peripheral blood mononuclear cells into the area of spinal cord injury of pigs in subacute period

P211

Rui Nobre *CNC, University of Coimbra*
Assessing cerebellar neurometabolic biomarkers by *in vivo* magnetic resonance spectroscopy in a transgenic mouse model of Machado-Joseph disease

POSTER SESSION 1 / 17 OCT GARDEN FLOOR 13.10 - 15.10

CNS AND OCULAR DISEASES

P213

Ludovico Arcuri *CNR National Research Council, Institute of Neuroscience, Milan*
AAV.PHP.eB-mediated OPA1 gene expression in a mouse model of Parkinson's disease as a valuable strategy for neuroprotection

P215

Corinne Kostic *University of Lausanne*
Gene therapy in Rho$^{-/-}$ mice, a model to validate rod-specific vectors and to identify biomarkers of irreversible function restoration

P217

Arpad Palfi *Trinity College Dublin*
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Joshua Bartoe *Michigan State University, East Lansing, MI*
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Ruslan Grishanin *Adverum Biotechnologies, Menlo Park, CA*
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Angela Gritti *SR-TIGET and San Raffaele Hospital, Milan*
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Gloria Gonzalez-Aseguinolaza *FIMA, Pamplona*
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Asha Recino *University of Cambridge*
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Massimiliano Caiazzo *Utrecht University*
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Francesco Saverio Tedesco *University College London*
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Manel Llado Santaeularia *TIGEM, Naples*
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John Zuris *Editas Medicine, Cambridge, MA*
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Thomas Kocher *EB House Austria, Medical University Salzburg*
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Marshall Huston *Sangamo Therapeutics, Richmond, CA*
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Afroditi Avgerinou *University College London*
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Beatrice Claudia Cianciotti *San Raffaele Scientific Institute, Milan*
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Nerea Zabaleta Lasarte *CIMA, IdiSNA, University of Navarra, Pamplona*
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Marisa Cappella *Sorbonne University, INSERM U974, AIM, Paris*
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Giulia Maule *CIBIO, University of Trento*
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Carsten Lederer *The Cyprus Institute of Neurology and Genetics*
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Dina Glazkova *Central Research Institute of Epidemiology, Moscow*
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Alessia Cavazza *Great Ormond Institute of Child Health, University College London*
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Nisha Nair *VUB, Brussels*
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Matias I. Autio *Genome Institute of Singapore*
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Maria Garcia Bravo *CIEMAT/CIBERER-ISCIII, Madrid*
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Yunus Emre *Eksi Akdeniz University Center for Gene and Cell Therapy, Antalya*
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Taeyoung Koo *Institute for Basic Science, Seoul*
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Antonio Casini *CIBIO, University of Trento*
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Jessica Carmen *MaxCyte, Gaithersburg, MD*
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Sonia Guedan Carrrio *The University of Pennsylvania, Philadelphia, PA*
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Eliana Ruggiero *San Raffaele Scientific Institute, Milan*
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Davide Grandolfo *Cell Therapy Catapult, London*
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Gabriele Antonarelli *SR-TIGET, Milan*
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Albert Rizvanov *Kazan Federal University*
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Yaling Shi *Lonza, Basel*
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Josefina Nilsson *Vironova, Stockholm*
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Valerie Kedinger *Polyplus Transfection, Illkirch*
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Randy Chandler *National Institutes of Health, Bethesda, MD*
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Fulvio Mavilio *Audentes Therapeutics, San Francisco, CA*
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Saori Miwa *Jikei University*
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Randy Chandler *National Institutes of Health, Bethesda, MD*
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Valentina Poletti *Dana-Farber Cancer Institute - Harvard Medical School, Cambridge, MA*
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Umut Cagin *Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*
Functional, biochemical and transcriptional rescue of advanced Pompe disease with liver expression of secretable GAA

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Giulia De Sabbata *ICGEB, Trieste*
Development of a novel AAV-based gene therapy in combination with tolerogenic nanoparticles for sustained treatment of ornithine transcarbamylase deficiency

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Sean Armour *Spark Therapeutics, Philadelphia, PA*
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Salih Sanlioglu *Gene and Cell Therapy Center of Akdeniz University, Antalya*
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Robert Kaiser *Children's Hospital of Minnesota, Minneapolis, MN*
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Sem Aronson *UMC University of Amsterdam*
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Sereina Deplazes *University Children's Hospital Zürich*
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Ruben van Helden *Leiden University Medical Center*
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Sem Aronson *UMC University of Amsterdam*
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Daniela Gnani *SR-TIGET, Milan*
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Ilya Grigoryev *Kazan Federal University*
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Angelina Titova *Kazan Federal University*
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Mikhail Mavlikeev *Kazan Federal University*
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Angelina Titova *Institute of Fundamental Medicine and Biology, KFU, Kazan*
Parenchymal and nonparenchymal cellular proliferation dynamics after partial hepatectomy with and without 2-acetylaminofluorene injection in rats

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Pavel Makarevich *Lomonosov Moscow State University*
Role of paracrine factors secreted by mesenchymal stromal cells in spermatogonial stem cell niche regulation

MUSCLE, CARDIOVASCULAR AND PULMONARY DISEASES

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Andrea Sarasin *Sarepta Therapeutics, Cambridge, MA*
PPMO treatment results in widespread muscle delivery and efficacy in mice and cynomolgus monkeys: a potential therapeutic platform for the treatment of Duchenne muscular dystrophy

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Alberto Malerba *Centre of Biomedical Sciences, School of Biological Sciences, Royal Holloway University of London*
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Angelina Titova *Kazan Federal University*
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Astrid Brull *Sorbonne University, Paris*
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Thayane Crestani *Laboratory of Genetics and Molecular Cardiology, Heart Institute, University of São Paulo Medical School*
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Patrice Vidal *Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*
AAV vector-based gene therapy for glycogen storage disease type III

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Fabien Dorange *Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*
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Tiziana Coradin *Oxford BioMedica*
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Camille Vaubourg *Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*
Evaluation in mice of two genetic modifiers of muscular dystrophy on the phenotypic severity of FKRP deficiency

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Albert Rizvanov *Kazan Federal University*
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Omar Dabbous *AveXis, Bannockburn, IL*
Indirect treatment comparison of AVXS-101 to nusinersen for the treatment of type 1 spinal muscular atrophy (SMA1)

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Antonietta Messina *INSERM UMR 1193, Villejuif*
Spheroid formation and evaluation of human induced pluripotent stem cell-derived hepatocytes through self-assembly process

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Ji Hyun Kim *Rznomics, Gwangju*
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Yen-Hui Chan *National Taiwan University Hospital, Taipei*
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Bryan Piras *St. Jude Children's Research Hospital, Memphis, TN*
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Bryan Piras *St. Jude Children's Research Hospital, Memphis, TN*
Optimization of transfection conditions to maximize AAV production in suspension 293-based cells

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Pavel Makarevich *Institute for Regenerative Medicine, Medical Research and Education Centre, Lomonosov Moscow State University*
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Omar Dabbous *AveXis, Bannockburn, IL*
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Omar Dabbous *AveXis, Bannockburn, IL*
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Caroline Odenwald *PROGEN Biotechnik, Heidelberg*
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Julie Chassagne *UMRS974, UPMC Sorbonne University, Inserm / AIM, Paris*
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Quentin Enjalbert *Anaquant, Villeurbanne*
A universal analytical tool for protein identification and quantification in gene or cell therapies development

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John Murray *University of Florida, Jacksonville, FL*
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Ekaterina Naumenko *Kazan Federal University*
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David Suter *École Polytechnique Fédérale de Lausanne*
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Louise Bullen *Manchester Metropolitan University*
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Sarah Le Saux *Institute Charles Gerhardt Montpellier, UMR 5253 CNRS-UM-ENSCM*
Exploring the potential of extracellular vesicles as drug delivery systems

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Matthias Bozza *DKFZ and NCT, Heidelberg*
A novel non-viral, non-integrative nano-DNA vector system for T-cell engineering

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Petr Ilyinskii *Selecta Biosciences, Watertown, MA*
Tolerogenic nanoparticles enhance transgene expression and intracellular vector copy number after initial and repeated administrations of AAV-based gene therapy vectors through immunological and non-immunological mechanisms

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Betty Maepa *University of the Witwatersrand, Johannesburg*
Application of a synthetic adeno-associated viral vector for the delivery of anti-hepatitis B virus gene therapeutics

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Alissa Bray *Oxford Genetics*
Reconfiguration of AAV Rep-Cap coding sequences significantly increases viral vector yield and enables inducible AAV production in HEK293 cells

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Youna Coquin *Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE*
New lentiviral pseudotypes based on murine syncytin-A or -B are efficient tools for the transduction of murine and human B cells

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Xiaoxia Shi *UMC University of Amsterdam*
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Claire Domenger *Heidelberg University Hospital*
Post-transcriptional fine-tuning of AAV vector gene expression for hemophilia A gene therapy

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Lori Karpes *Voyager Therapeutics, Cambridge, MA*
Challenges and successes in high concentration AAV drug product formulation development

P453

Heidi Hynynen *Kuopio Center for Gene and Cell Therapy*
Development of AAV full/empty capsid ratio analysis using semi-automated transmission electron microscopy

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Diana Schenkwein *A.I.Virtanen Institute, University of Eastern Finland, Kuopio*
Protein transduction of genome editing enzymes with new lentivirus vector derived particles

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Giuliana Vallanti *MolMed, Milan*
Lentiviral/retroviral vector large scale manufacturing

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Jens Gruber *German Primate Center, Göttingen*
Improving viral and non-viral vectors: adopting exosome biology for efficiency and hydrogels for controlled release

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Ram Shankar *PlasmidFactory, Bielefeld*
AAV vectors are going viral in gene and cell therapy

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Kerstin Hein *Cevec Pharmaceuticals, Cologne*
Generation of helper virus-free adeno-associated viral vector packaging/producer cell lines based on a human suspension cell line

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Kyung-Ju Choi *CHA Bundang Medical center, CHA University*
Anti-tumor activity of GM-CSF and IL-12 expressing oncolytic HSV-1

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Jeffrey Slack *Voyager Therapeutics, Cambridge, MA*
Production of Caltech AAV capsids with BEVS: challenges and solutions

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Pavel Makarevich *Lomonosov Moscow State University*
Development of a plasmid construct encoding HGF and VEGF165 for gene therapy

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Lionel Galibert *Kuopio Center for Gene and Cell Therapy*
Large-scale optimisation and production of rAAV vector encoding VEGF in iCELLis bioreactors

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Simon Chanas *GSK, Stevenage*
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Marco Schmeer *PlasmidFactory, Bielefeld*
Minicircle DNA as starting material for development of ATMPs

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Andre Sobczyk *Brain and Spine Institute, Paris*
iVector, a core facility for bioproduction of viral vectors (lentivirus, AAV & CAV-2) used in neurosciences research at the Brain and Spine Intitute

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Pascale Belguise *Polyplus Transfection, Illkirch*
Addressing large-scale manufacturing of clinical grade viral vectors using an optimized PEI-based transfection process

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Roland Leathers *Thermo Fisher Scientific, Waltham, MA*
A novel suspension-based lentiviral production platform to achieve cost-effective clinical manufacturing

P483

Weiheng Su *University of Oxford*
Exploiting adenovirus mechanisms for the enhanced production of AAV vectors

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Pasqualina Colella *Genethon, Evry, UMR_S951, Inserm, Univ Evry, University Paris Saclay*
Tandem promoter design confers tolerogenic and persistent transgene expression to AAV gene therapy in neonate Pompe mice

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Simone Merlin *University of Piemonte Orientale, Novara*
FVIII expression driven by its native promoter allowed phenotypic correction in hemophilic mice

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Anna Kajaste-Rudnitski *SR-TIGET, Milan*
Cyclosporine H overcomes IFITM3-mediated innate immune restriction to lentiviral transduction and gene editing in human haematopoietic stem cells

P491

Petr Ilyinskii *Selecta Biosciences, Watertown, MA*
Combination of an engineered AAV vector Anc80 and tolerogenic nanoparticles encapsulating rapamycin enables efficient transgene expression in mice with pre-existing neutralizing antibodies and provides a therapeutic benefit in a mouse model of methylmalonic acidemia

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Sylvain Cecchini *Voyager Therapeutics, Cambridge, MA*
Engineering of 5' UTR to control the expression and incorporation level of VP1 during rAAV vector production using a baculovirus system

P006

Mohamed Zoughaib *Kazan Federal University*
Development of nanosized hydroxyapatite-containing cryogels as a matrix for osteogenic cells

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Omid Mashinchian *Nestlé Institute of Health Sciences, Lausanne*
3D-derivation of uncommitted human muscle stem cells from iPSCs

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Ekaterina Naumenko *Kazan Federal University*
Effects of curcumin-loaded halloysite on C.elegans nematodes in host-microbiome interactions

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Ekaterina Naumenko *Kazan Federal University*
The mechanism of the nanoparticles penetration into human cells

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Mohamed Zoughaib *Kazan Federal University*
Characterization of microelement-doped hydrogels as a bioactive cellular matrix

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Mohamed Zoughaib *Kazan Federal University*
In vitro optimization of a cryogel based tumour model

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Ekaterina Naumenko *Kazan Federal University*
Magnetic nanomaterials for 3D spheroids formation

BLOOD DISORDERS

P024

Aphrodite Georgakopoulou *G.Papanicolaou Hospital, Thessaloniki*
Reversal of the thalassemic phenotype in mice post *in vivo* transduction of mobilized hematopoietic stem cells (HSCs) with an integrating hybrid adenovirus vector system

P026

Wolfgang Miesbach *University Hospital, Frankfurt*
Surgery and bleed management in patients receiving AMT-060 in a Phase I/II trial: evaluation of the safety of exogenous FIX treatment after gene transfer

P028

Maria Ester Bernardo *San Raffael Scientific Institute, Milan*
Impairment in the hematopoietic supportive capacity of bone marrow stroma in beta-thalassemia patients is associated with niche iron overload and oxidative stress

P030

Gülen Güney *Hacettepe University, Anuara*
The NPY-Y1 receptor for neuropeptide Y is highly expressed on hematopoietic stem cells from bone marrow and cord blood

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P032

Luca Basso-Ricci *SR-TIGET, Milan*
Kinetics and quality assessment of mobilized stem cell product upon G-CSF and Plerixafor administration in patients with hematological disorders for gene therapy approaches

P034

Aphrodite Georgakopoulou *G.Papanicolaou Hospital, Thessaloniki*
Efficient *ex vivo* gene transfer of γ-globin in human thalassemic $CD34^+$ cells using an integrating hybrid adenoviral vector system

P036

Diego Leon-Rico *University College London Institute of Child Health*
Systematic comparison of culture media and transduction enhancers for optimised $CD34^+$ cell-based retroviral gene therapy protocols

P038

Kavitha Rajavel *Shire, Dublin*
The prevalence of pre-existing humoral immunity to AAV in adults with severe haemophilia: interim results from an ongoing global epidemiology study

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Jose Antonio Casado Olea *CIEMAT/ CIBERER-ISCIII, Madrid*
The *ex vivo* transduction of human hematopoietic stem cells induces the expression of NKG2D ligands

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Sophie Ramadier *Imagine Institute, INSERM UMR1163, Paris*
Combination of lentiviral and genome editing technologies for the treatment of sickle cell disease

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Sara Deola *Sidra Medicine, Qatar*
Flow-cytometry platform for intracellular detection of FVIII in blood cells: a new tool to assess gene therapy efficiency for haemophilia-A

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Akbar Farjadfar *Fasa University of Medical Sciences*
Gene therapy of breast cancer related anemia by delivering of erythropoietin in mice model

P048

Sergei Abramov *Kazan Federal University*
The risk allele A of rs200395694 associated with SLE in Swedish patients affects on MEF2D gene regulation and alternative splicing

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Mariya Tikhomirova *Kazan Federal University*
The role of autophagy expression in T-lymphocytes of patients with severe asthma

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Carlos Carrascoso-Rubio *CIEMAT/ CIBERER-ISCIII, Madrid*
Towards the gene therapy of the bone marrow failure in patients with dyskeratosis congenita

P054

Elena Barbon *Genethon, UMR_S951 Inserm, Univ Evry, University Paris Saclay, EPHE*
The potential use of nanobodies delivered via AAV vectors in the treatment of haemophilia

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Thomas Hartjes *Dept. of Neurosurgery, Erasmus MC, Rotterdam*
Organotypic multicellular spheres (OMS) as a 3D model system to study oncolytic adenovirus responses in glioblastoma tumours

P062

Akbar Farjadfar *Fasa University of Medical Sciences*
Evaluation of proapoptotic effects of MSC expressing endostatin and TRAIL on SVEC and 4T1 cell lines and mouse model

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Ai-Li Shiau *National Cheng Kung University, Tainan*
Adenovirus-mediated transfer of shRNA against Elovl6 reduces the progression of hepatocellular carcinoma

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Filippo Birocchi *San Raffaele University, Milan*
Development of a chimeric form of IFNa for "on demand" *in vivo* cancer gene therapy

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Hrvoje Miletic *Haukeland University Hospital, Bergen*
HSV-tk mediated suicide gene therapy leads to an immunogenic cell death of glioblastoma cells and a T-cell mediated immune response

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Pierre Cordelier *Inserm U1037, Toulouse*
H-1 parvovirus inhibits both primary tumor and metastatic growth of human pancreatic tumours

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Sung Soo Kim *Department of Anatomy, Ajou University School of Medicine, Suwon*
Synergistic antitumour effects of temozolomide in combination with suicide gene expressing mesenchymal stem cells in orthotopic xenograft glioma models

P074

Pedro Leite *Azevedo Stem Cell Laboratory, Bone Marrow Transplantation Unit, National Cancer Institute (INCA), Rio de Janeiro*
WNT signaling pathway regulates Bmp4 expression in mesenchymal stromal cells from acute myeloid leukemia patients

P076

Chang Ho Lee *Dankook University, Yongin*
Gene replacement based on RNA reprogramming as an effective approach for personalized cancer theranostics

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Mark Shearman *AGTC, Cambridge, MA*
Comparable efficiency of photoreceptor transduction by AAV2tYF, AAV5 and AAV8 in non-human primates

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VECTOR DEVELOPMENT

P442

Jessika Ceiler *DKFZ and NCT, Heidelberg*
Novel insights into wild-type and recombinant AAV mitochondrial integration

P444

Pedro E Cruz *University of Florida, Gainsville, FL*
Evaluating central nervous system promoters using double strand adeno-associated virus 6 triple mutants (T492V-Y705F-Y731F) virus

P446

Izabela Kraszewska *Jagiellonian University, Krakow*
Recruitment of MRN complex to the ITRs limits permissivity of the cells to scAAV transduction

P448

Adrian Westhaus *Children's Medical Research Institute, Westmean, NSW*
The influence of AAV shuffled library platform design on the outcome of directed evolution

P450

Giridhara R Jayandharan *Indian Institute of Technology (IIT) Kanpur*
Deciphering the role of hepatic cell microRNAome during AAV infection

P452

Albert Rizvanov *Kazan Federal University*
Adenoviral-mediated lactoferrin gene therapy for abscesses of the maxillofacial area in rat model

P454

Bas Bosma *uniQure, Amsterdam*
Comprehensive comparative evaluation of the qualitative attributes of AAV5 batches produced in mammalian and insect cells

VECTOR DEVELOPMENT

P456

Kristoffer Riecken *University Medical Centre Hamburg-Eppendorf, Hamburg*
Efficient pseudotyping of different retroviral vectors using a novel, codon-optimised gene for chimaeric GALV envelope

P458

MoonKyung Kang *Chungnam National University, Daejeon*
Human codon-optimization of GaLV-pseudotyped semi-retroviral replicating vector system improved genomic stability and anti-cancer activity

P460

Tom Payne *Oxford Genetics*
Generation and characterisation of cGMP-compliant suspension HEK293-TetR cell line for adenoviral vaccine manufacture

P462

Gloria Gonzalez-Aseguinolaza *FIMA, Pamplona*
Analysis of the impact of NAbs on the transduction efficiency of the synthetic hepatotropic AAV-Anc80 vector in non-human primates

P464

Win Cheung *REGENXBIO., Rockville, MD*
Relative infectivity as a reliable method for quantifying differences in the infectivity of AAV vectors *in vitro*

P466

Qian Liu *Oxford Genetics*
Generation of cGMP-compliant stable packaging and producer cell lines for inducible lentiviral vector production

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VECTOR DEVELOPMENT

P468

Zsuzsanna Izsvak *MDC, Berlin*
While, no barrier insulator function is required for stable transgene expression from Sleeping Beauty transposon, 6CTCF enhancer blocker improves its biosafety for therapeutical applications

P470

Alisa Nousiainen *A.I.Virtanen Institute, University of Eastern Finland, Kuopio*
Inclusion of HR arms into LVs improves rDNA-directed integration with an IN-I-PpoIN119A fusion protein

P472

Maria Ontiveros *Autonomous University of Barcelona*
UPV: production of customized high quality viral vectors at a joint UAB-VHIR technological platform

P474

Friedrich Koch-Nolte *University Medical Center Hamburg-Eppendorf*
Using nanobodies to target AAV vectors to specific cells

P476

Albert Rizvanov *Kazan Federal University*
Construction of plasmid vectors encoding pro-angiogenic factors for gene therapy using picornoviral structural elements

P478

Yuanhong Li *uniQure, Amsterdam*
uniQure down stream purification process shows excellent viral clearance capabilities

P480

Albert Rizvanov *Kazan Federal University*
No evidence of ectopic bone formation in soft tissues of rats injected with plasmid DNA encoding human VEGF165 and BMP2 cDNA

VECTOR DEVELOPMENT

P482

Chiara Crosta *Anemocyte, Gerenzano*
Analytical characterization of plasmid DNA for viral vector

P484

Jihad EL Andari *University Hospital Heidelberg*
Identification of new muscle-tropic adeno-associated virus (AAV) capsids for treatment of rare hereditary muscular disorders

P486

Juan Manuel *Iglesias Gonzalez Synpromics Ltd.*
A novel genomics-based platform for the creation of synthetic promoters for cell and gene therapy

P488

Eduard Ayuso *INSERM UMR1089, University of Nantes*
MicroRNAs, overlooked contaminants of AAV vector batches

P490

Anne-Kathrin Herrmann *Heidelberg University Hospital, Cluster of Excellence CellNetworks, Heidelberg*
DNA family shuffling has no detrimental impact on the functionality of the assembly-activating protein (AAP) from AAV

P492

Michela Milani *SR-TIGET, Milan*
Liver-directed gene therapy in newborn mice with lentiviral vectors

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EUROPEAN SOCIETY OF GENE & CELL THERAPY

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

We achieve this in part through scientific and educational activities, in particular through measures aimed at the promotion and the exchange of information and ideas with regard to gene therapy, cell therapy, genetic vaccination, the encouragement of research fields, and clinical applications. As such we would like to support the activities of national societies that share this goal – please see below for information and contact details of some national societies to help you, should you wish to get in touch with any of them.

UNITED KINGDOM

British Society for Gene Therapy

office@bsgct.org.uk
For further information and future events:
www.bsgct.org

GERMANY

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

dggt.office@dg-gt.de
For further information and future events:
www.dg-gt.de

FINLAND

SUOMEN GEENITERAPIASEURA
FINNISH SOCIETY OF GENE THERAPY

For further information, contact details and future events:
www.fsgt.fi

FRANCE

Société Française de Thérapie Cellulaire et Génique

office@sftcg.fr
For further information and future events:
www.sftcg.fr

GREECE

HELLENIC SOCIETY OF GENE THERAPY AND REGENERATIVE MEDICINE

info@generegther.gr
For further information and future events:
www.generegther.gr/en/

THE NETHERLANDS

For further information, contact details and future events:
www.nvgct.nl

SPAIN

Sociedad Española Terapia Génica y Celular

office@setgyc.es
For further information and future events:
www.setgyc.es

Autumn 2019: Leipzig

Spring 2020: Hannover

Spring 2021: Munich

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

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Société Française de Thérapie Cellulaire et Génique

The SFTCG wants to function as a platform to:

1. Encourage the communication between scientists in the field of gene and cellular therapy through the organization of its yearly meeting and exchange between labs in of students and novel technologies.
2. And provide and interface between scientist, clinicians and patients
3. Inform in a comprehensive and responsible way the general public about gene and cellular therapy in France and Europe

BECOME A MEMBER AND...

...you will benefit from heavily discounted fees for SFTCG events. In addition, you will have a unique chance to enter the "who is who" online database of gene and cellular therapy labs in France. You will have full access to job and grant proposals, but also to up-to-date news and views on gene and cellular therapy programs, clinical trials and regulatory affairs, links to journals in the field and particularly to Human Gene Therapy journal family. This will also be a unique occasion to network with expert scientists and clinicians, to grow your professional network, expand business opportunities, and advance your career in the field of gene and cell therapy.

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