PROGRAMME 19 OCTOBER 2021

13h45 - 14h00 REGISTRATION

Plenary 1: Opening Session

Studio 1 14h00 - 16h30

Chairs: *Hildegard Büning, Hannover Medical School*

Thierry VandenDriessche, VUB Brussels

14h00 **Hildegard Büning**
ESGCT President, Hannover Medical School
Welcome

14h10 **Thierry VandenDriessche**
VUB Brussels
Opening words

14h15 **Ugur Sahin**
BioNTech, Mainz
INV02

15h00 **Mark Kay**
Stanford University
INV03: Why do rAAV vectors show discordant transduction properties between species?

15h45 **Drew Weissman**
University of Pennsylvania
INV04: Nucleoside-modified mRNA-LNP therapeutics

16h30 - 16h55 BREAK

Parallel 1a: CNS & sensory I

Studio 1

16h55 - 19h00

Chairs: *Stylianos Michalakis, University of Munich*

Deniz Dalkara, Institut de la Vision, Paris

17h00 **Deniz Dalkara**
Institut de la Vision, Paris
INV05: Early to late stage gene therapy interventions for inherited retinal degenerations

17h33 **Manny Simons**
Akouos, Boston MA
INV06: Inner ear conditions: Development of precision genetic medicines in a new therapeutic area

18h06 **Wuh-Liang Hwu**
PTC Therapeutics, Inc
OR01: Eladocagene exuparvovec improves body weight and reduces respiratory infections in patients with aromatic l-amino acid decarboxylase deficiency

18h22 **Patrizia Tornabene**
Tigem, Naples
OR02: Inclusion of a degron reduces the levels of undesired inteins after AAV-mediated protein trans-splicing in the retina

18h40 **Patrick Yu Wai Man**
University of Cambridge
OR03: The phase III REFLECT trial: Efficacy and safety of bilateral gene therapy for Leber Hereditary Optic Neuropathy (LHON)

Parallel 1b: AAV as a gene therapy tool from virus to vector

Studio 2

16h55 - 19h00

In memoriam - Mavis Agbandje-McKenna

Chairs: *Guangping Gao, University of Massachusetts*

Aravind Asokan, Duke University School of Medicine

17h00 **Dirk Grimm**
Heidelberg University
INV08: Faster, higher, stronger - a short history of record-breaking AAV vector technologies

17h30 **Hildegard Büning**
Hannover Medical School
INV09: Filling the pipeline - the next generation of AAV vectors for gene therapy

18h00 **Guangping Gao**
University of Massachusetts
INV10: Potential roadblocks to clinical translation and commercialization of AAV gene Tx: Assessment and mitigation strategies

18h30 **Aravind Asokan**
Duke University School of Medicine
INV11: Engineering AAV biology

Parallel 1c: CAR T & CAR NK cells

Studio 3

16h55 - 19h00

Chairs: *Hinrich Abken, Universitätsklinikum Regensburg*

Dirk Nettelbeck, DKFZ, Heidelberg

17h00 **Michael Hudecek**
Universitätsklinikum Würzburg
INV12: New targets and technologies for CAR T cell immunotherapy

17h26 **Winfried Wels**
Georg Speyer Haus, Frankfurt
INV13: CAR-engineered NK cells as allogeneic off-the-shelf therapeutics

17h58 **Alessio Nahmad**
Tel Aviv University
OR05: Frequent aneuploidy in primary human T Cells following CRISPR-Cas9 cleavage

18h14 **Jonas Kath**
Charité University, Berlin
OR06: Improved non-viral CAR-reprogramming of human T cells using CRISPR-Cas and double-stranded DNA

18h30 **Di Yu**
Uppsala University
OR07: Expression of a pathogenic virulence factor enhances the efficacy of CAR-T cell therapy against solid tumors

18h45 **Matteo Doglio**
San Raffaele Scientific Institute, Milan
OR08: CAR-Tregs for the treatment of Systemic Lupus Erythematosus

19h00 - 19h30 BREAK

PROGRAMME 19 OCTOBER 2021

BREAK	Sponsor Symposium	BREAK
Studio 1	Studio 2 — MaxCyte 19h30 - 20h15 *Chairs:* *Chris Mann, MaxCyte*	Studio 3
	19h35 **Stephen Schoenberger** *La Jolla Institute for Immunology, UCSD Moores Cancer Center* **MAXC01: Exploring the role of NeoAg-specific CD4+ T cells in cancer immunotherapy**	

PROGRAMME 20 OCTOBER 2021

08h30 - 08h55 REGISTRATION

Parallel 2a: Primary immunodefficiencies	Parallel 2b: Gene editing I	Parallel 2c: Immunotherapy for cancer & CAR T cells
Studio 1 08h55 - 11h01 *Chairs:* *Rafael Yañez, Royal Holloway, London* *Robyn Bell, Imperial College London*	Studio 2 08h55 - 11h00 *Chairs:* *Adi Barzel, Tel Aviv University* *Ayal Hendel, Bar-Ilan University, Tel Aviv*	Studio 3 08h55 - 11h00 *Chairs:* *José Antonio Pérez-Simón, Institute of Biomedicine of Sevilla* *Felipe Prósper, University of Navarra*
09h00 **Elena Almarza** *Rocket Pharma* **INV14: Gene Therapy for Leukocyte Adhesion Deficiency Type I (LAD-I): A 12-year journey**	09h00 **Jacob Corn** *ETH Zurich* **INV17: Hematopoietic stem cell re-quiescence balances stemness and HDR during genome editing**	09h00 **Sonia Guedan** *IDIBAPS, Barcelona* **INV19: Increasing the therapeutic index of CAR-T cells in solid tumors**
09h30 **Karin Pike-Overzet** *Leiden University* **INV15: Bringing lentiviral gene therapy to clinical application for RAG1-SCID**	09h30 **Adi Barzel** *Tel Aviv University* **INV18: In vivo engineered B cells retain memory and secrete high titers of anti-HIV antibodies in mice**	09h30 **Bart Neyns** *UZ Brussels* **INV20: Exploring the potential of dendritic cell therapy for the treatment of advanced melanoma, from moDC to myDC**
10h00 **Giorgia Santilli** *University College London* **INV16: Genome editing in human hematopoietic stem cells for the treatment of X-linked agammaglobulinemia**	10h00 **Daniela Benati** *University of Modena* **OR11: CRISPR-mediated genome editing to redirect T cells against Non-Small Cell Lung Cancer**	10h00 **Beatrice Claudia Cianciotti** *San Raffaele Scientific Institute, Milan* **OR15: Tim-3, LAG-3 and 2B4 disruptions differentially regulate anti-tumor response of TCR gene edited memory stem T cells**
	10h15 **Sean Burns** *Intellia Therapeutics* **OR12: Consecutive genome editing in non-human primate achieves durable production of human alpha-1 antitrypsin at physiologic levels and reduction of the homologous native protein**	10h15 **Robert Hawkins** *Instil Bio* **OR16: Clinical feasibility and treatment outcomes with unselected autologous tumour-infiltrating lymphocyte (TIL) therapy in patients with advanced cutaneous melanoma**
10h30 **Claire Booth** *University College London* **OR09: A phase 1/2 study of lentiviral-mediated ex-vivo gene therapy for pediatric patients with severe leukocyte adhesion deficiency-I (LAD-I): Interim results**	10h30 **Aboud Sakkal** *Genethon, Evry* **OR13: MMEJ-mediated IDLV knock-in via CRISPR/Cas9 in human hematopoietic stem/progenitor cells**	10h28 **María Tristán Manzano** *Genyo, Granada* **OR17: Physiological (TCR-like) regulated lentiviral vectors for the generation of improved CAR-T cells**
10h45 **Christopher Riling** *Spark Therapeutics* **OR10: Investigational liver gene transfer of C1-INH for the treatment of Hereditary Angioedema**	10h45 **Viviane Dettmer-Monaco** *University of Freiburg* **OR14: Preclinical development of TALEN®-based genome editing in T cells for the treatment of Hyper-IgE-Syndrome**	10h45 **Yong Zhang** *Intellia Therapeutics* **OR18: A novel strategy for off-the-shelf T cell therapies evading host T cell and NK cell rejection**

11h01 - 11h25 BREAK

Plenary 2: Advances in preclinical studies

Studio 1 11h25 - 13h33

Chairs: *Vincenzo Cerullo, University of Helsinki*

Claire Booth, University College London

11h30 **Luigi Naldini**
SR Tiget, Milan
INV21: Tumor targeted delivery of interferon by HSC gene therapy reprograms the glioblastoma microenvironment toward protective immunity: from preclinical modeling to clinical testing

12h00 **Gloria Gonzalez-Aseguinolaza**
University of Navarra, Pamplona
INV22: Gene therapy for liver inherited diseases

12h31 **Alessia Cavazza**
University College London
INV23: Preclinical studies for a hematopoietic stem cell gene editing platform to treat Wiskott-Aldrich Syndrome

13h02 **Len Seymour**
University of Oxford
INV24: Using oncolytic viruses to mediate HLA-independent immunotherapy

BREAK

Studio 1
13h33 - 15h10

Sponsor Symposium: Pioneering in gene therapies: sharing our experience with AADC deficiency

Studio 2
13h45 - 14h45

Chairs: *Ellen Welch, PTC Therapeutics*

13h46 **Matthew Klein**
PTC Therapeutics
PTC01: Overcoming barriers: The approach to gene therapy at PTC Therapeutics

Agathe Roubertie
Hôpital Gui de Chauliac, Montpellier
PTC02: AADC deficiency disease overview and current management strategies

Wuh-Liang Hwu
National Taiwan University Hospital, Taipei
PTC03: Intraputaminal gene therapy for AADC deficiency: Clinical updates

Sponsor Symposium: AVRO: Innovations in ex vivo lentiviral gene therapy expand clinical opportunities and enhance safety

Studio 3
13h45 - 14h45

Chairs: *David Williams, Boston Children's Hospital*

13h46 **Axel Schambach**
Hannover Medical School
AVR01: From basic biology to clinical application: Impact on safety and clinical scope

Chris Mason
AvroBio
AVR02: Safety: Real-world experience from lysosomal disorder clinical trials

Luca Biasco
AvroBio
AVR03: Platform: Potency, fate and stability of infused cells

14h45 - 15h10 BREAK

PROGRAMME 20 OCTOBER 2021

Parallel 3a: Liver & metabolic diseases I

Studio 1
15h10 - 17h15

Chairs: *Nicola Brunetti-Pierri, Tigem, Naples*
Terry Flotte, University of Massachusetts

15h15	**Alberto Auricchio**	*Tigem, Naples* **INV25: AAV-mediated liver gene therapy for mucopolysaccharidosis VI**
15h42	**Charles Venditti**	*NIH, NHGRI, Bethesda, MD* **INV26: New genomic therapies to treat methylmalonic acidemia (MMA)**
16h15	**Jolanda Liefhebber**	*UniQure* **OR19: Pre-clinical proof of concept of an AAV5-GLA gene therapy for Fabry disease resulting in cross-correction in GLA-KO mice and non-human primates in target organs**
16h30	**Tarekegn Geberhiwot**	*University of Birmingham* **OR20: AAV8 gene therapy as a potential treatment for adults with late-onset ornithine transcarbamylase (OTC) deficiency: updated results from a phase 1/2 clinical trial**
16h46	**Yvonne Aratyn-Schauss**	*Beam Therapeutics* **OR21: In vivo base-editing corrects metabolic defects in Glycogen Storage Disease type-Ia**
17h00	**Agnese Padula**	*Tigem, Naples* **OR22: AAV-mediated gene therapy for Wilson disease using split-intein technology**

Parallel 3b: Cardiovascular & lung diseases

Studio 2
15h10 - 17h15

Chairs: *Annakaisa Tirronen, University of Eastern Finland, Kuopio*
Seppo Ylä-Herttuala, University of Eastern Finland, Kuopio

15h15	**John Engelhardt**	*University of Iowa* **INV27: Utility of genetic ferret models for developing Cystic Fibrosis gene and cell therapies**
15h45	**Silva Priori**	*University of Pavia* **INV28: Gene Therapy to the heart: Challenges and progresses**
16h15	**Seppo Ylä-Herttuala**	*University of Eastern Finland, Kuopio* **INV29: Cardiac gene therapy with VEGF-B**
16h45	**Miriam Hetzel**	*Hannover Medical School* **OR23: Exchange of alveolar macrophages with ex vivo generated macrophages restores pulmonary immunity by niche specific adaption**
17h01	**Nora Clarke**	*Imperial College London* **OR24: Ex vivo transduced macrophages produce therapeutic levels of secreted protein when transplanted to the lung**

Parallel 3c: Muscle diseases

Studio 3
15h10 - 17h17

Chairs: *Capucine Trollet, Centre de Recherche en Myologie, UMRS 974*
Serge Braun, AFM, Paris

15h15	**Charles Gersbach**	*Duke University, School of Medicine, NC* **INV30: Genome editing for the treatment of Duchenne Muscular Dystrophy**
15h45	**Isabelle Richard**	*Genethon, Evry* **INV31: Gene replacement therapy in LGMD-R1**
16h15	**Perry Shieh**	*UCLA* **INV32: ASPIRO gene therapy trial in X-linked myotubular myopathy (XLMTM): update on preliminary efficacy and safety findings**
16h45	**Laura Lombardi**	*Tenaya Therapeutics* **OR25: Prevention of premature lethality and reversal of cardiac hypertrophy with an optimized MYBPC3 gene therapy**
17h00	**Fanny Collaud**	*Genethon, Evry* **OR26: Artificial miRNA-mediated glycogen synthase silencing as effective substrate reduction therapy in glycogen storage diseases mouse models**

17h15 - 17h40 BREAK

Plenary 3: Towards innovative gene therapy trials

Studio 1 17h40 - 19h45

Chairs: *Alessandro Aiuti, SR Tiget, Milan*
Gloria Gonzalez- Aseguinolaza, University of Navarra, Pamplona

17h45	**Alessandra Biffi**	*University of Padova, Harvard Stem Cell Institute* **INV33: Innovative HSC gene therapy approaches for neurodegenerative diseases**
18h15	**Chiara Bonini**	*Ospedale San Raffaele, Milan* **INV34: Genome editing for cancer immunotherapy**
18h45	**Selim Corbacioglu**	*University Hospital Regensburg* **INV35: Gene editing in hemoglobinopathies**
19h15	**José-Alain Sahel**	*Institut de la Vision, Paris* **INV36: Partial recovery of visual function in a blind patient after optogenetic therapy for non-syndromic Retinitis Pigmentosa**

PROGRAMME 21 OCTOBER 2021

08h30 - 08h55 REGISTRATION

Parallel 4a: CNS & sensory II

Studio 1

08h55 - 11h00

Chairs: *Nathalie Cartier, Institut de la Moëlle et du Cerveau, Paris*

Alberto Auricchio, Tigem Naples

09h00 Nathalie Cartier
Institut de la Moëlle et du Cerveau, Paris
INV37: Pathway Gene Therapy for Neurodegenerative Diseases : from rare to complex diseases.... and back

09h30 Maria José de Castro López
Hospital Clínico Universitario de Santiago de Compostela
INV38: Interim results of Transpher A, a multicentre, single-dose, Phase 1/2 clinical trial of ABO-102 investigational gene therapy for Sanfilippo syndrome type A (mucopolysaccharidosis IIIA)

10h00 Michael Hocquemiller
Lysogene, Paris
OR27: Intracisternal delivery of an AAV gene therapy candidate for the treatment of GM1 Gangliosidosis

10h15 Erika De Boever
Sio Gene Therapy
OR28: Phase 1/2 Trial of AXO-AAV-GM1 Gene Therapy for the Treatment of Infantile- and Juvenile-onset GM1 Gangliosidosis

10h30 Davide Sala
SR Tiget, Milan
OR29: Therapeutic advantage of combined gene/cell therapy strategies in the murine model of Sandhoff disease

10h45 Toloo Taghian
University of Massachusetts
OR30: Bicistronic AAV gene therapy for Tay-Sachs and Sandhoff diseases in a sheep model

Parallel 4b: Gene editing II

Studio 2

08h55 - 11h01

Chairs: *Rob Collin, Radboud University Medical Center, Nijmegen*

Massimiliano Caiazzo, Utrecht University

09h00 Anna Villa
SR Tiget Milan
INV40: Genome editing as novel therapeutic strategy to treat Recombination Activating Gene 1 (RAG1) Immunodeficiency

09h32 Manuel Gonçalves
Leiden University Medical Center
INV41: Expanding genome-editing tool delivery and targeting range in human cells

10h00 Aurélie Bedel
University of Bordeaux
OR31: CRISPR-Cas9 globin editing can induce megabase-scale partial uniparental disomy with imprinting defects in hematopoietic cells

10h15 Eric Pierce
Massachusetts Eye and Ear, Boston
OR32: BRILLIANCE: A Phase 1/2 single ascending dose study of EDIT-101, an in vivo CRISPR gene editing therapy, in CEP290-related retinal degeneration

10h30 Chikdu Shivalila
Wave Life Science
OR33: A versatile platform for ADAR-mediated RNA editing in vivo in preclinical models

10h46 Manuel Rhiel
Freiburg University
OR34: Preclinical development of a TALEN®-based genome editing therapy for RAG1 deficiency

Parallel 4c: Hematopoietic & bleeding disorders I

Studio 3

08h55 - 11h00

Chairs: *Giuliana Ferrari, SR Tiget, Milan*

Juan Bueren, Ciemat, Madrid

09h00 Wolfgang Miesbach
Goethe University Hospital, Frankfurt
INV42: Current update of clinical trials on gene therapy for haemophilia

09h30 Alessio Cantore
SR Tiget, Milan
INV43: Lentiviral gene therapy to the liver for hemophilia and beyond

10h00 Julian Sevilla
Hopsital Niño Jesus, Madrid
OR35: Gene therapy for Fanconi anemia [group a]: Interim results of RP-L102 clinical trials

10h15 John Lydeard
Vor Pharma
OR36: Pre-clinical evaluation, including genomic off-target analysis, of VOR33: a clinic-ready CRISPR/Cas9 engineered hematopoietic stem cell transplant for the treatment of acute myeloid leukemia

10h30 Federica Esposito
Tigem, Naples
OR37: AAV-intein mediated Factor VIII trans-splicing for gene therapy of Haemophilia A

10h45 Michela Lisjak
ICGEB, Trieste
OR38: Promoterless gene targeting approach combined with CRISPR/Cas9 efficiently corrects hemophilia B phenotype in neonatal mice

11h00 - 11h25 BREAK

Plenary 4: Presidential Symposium & Awards

Studio 1 11h25 - 14h00

Chairs: *Hildegard Büning, Hannover Medical School*

Juan Bueren, CIEMAT, Madrid

11h30 Hildegard Büning
Hannover Medical School
INV44: Presidential address

12h00 Hans Clevers
Utrecht University
INV45: Organoids to model human diseases

12h30 Else Kröner Fresenius Award
Alessandro Aiuti
SR Tiget, Milan
INV46: Haematopoietic stem cell gene therapy for inborn errors: Turning blood (stem) cells into a medicine

13h00 ESGCT Outstanding Achievement Award
Fulvio Mavilio
University of Modena
INV47: The present and future of genetically modified hematopoietic stem cells

13h30 ESGCT Young Investigator Award
Maria Ester Bernardo
SR Tiget, Milan
INV59: Hematopoietic Stem & Progenitor Cell Gene Therapy for Hurler Syndrome: interim clinical results and bone remodelling mechanisms

14h00-14h15 BREAK

PROGRAMME 21 OCTOBER 2021

BREAK

Studio 1

Sponsor Symposium: Innovations in AAV for Gene therapy

Studio 2
14h15 - 15h15

ThermoFisher SCIENTIFIC

Chairs: *Celine Martin, Thermo Fisher Scientific*

14h17 **Sofia Fernandes**
iBET, Oeiras
THE01: Benefits of using a media panel to address the diversity of HEK293 cell lines

Chao Yan Liu
Thermo Fisher Scientific
THE02: Scalable, high-titer, simplified AAV production in the AAV-MAX helper free AAV Production System

Jessica Tate
Thermo Fisher Scientific
THE03: GMP viral manufacturing – standardizing and scaling manufacturing processes

BREAK

Studio 3

15h15 - 15h40 BREAK

Parallel 5a: Non coding RNA

Studio 1
15h40 - 17h45

Chairs: *Zoltan Ivics, Paul Ehrlich Institute, Langen*
Sandro Banfi, Tigem, Naples

15h45 **Thomas Thum**
Hannover Medical School
INV50: Transformative RNA therapeutics for cardiovascular disease

16h15 **Stefanie Dimmeler**
Goethe University, Frankfurt
INV48: RNA Therapeutics in cardiovascular disease

16h40 **Sandro Banfi**
Tigem, Naples
INV49: Modulation of microRNA expression: A new therapeutic avenue for inherited retinal disease?

Parallel 5b: Gene editing III

Studio 2
15h40 - 17h45

Société Française de Thérapie Cellulaire et Génique SFTCG

Chairs: *Mario Amendola, Genethon, Evry*
Annarita Miccio, Institut Imagine, Paris

15h45 **Els Verhoeyen**
University of Nice, ENS Lyon
INV51: Baboon envelope pseudotyped "Nanoblades" carrying Cas9/gRNA complexes allow efficient genome editing and gene knock-in in hematopoietic cells and organoids

16h15 **Rodolfo Murillas**
Ciemat, Madrid
INV52: Genome editing strategies for genodermatoses therapy

16h45 **Anastasia Conti**
SR Tiget, Milan
OR39: Cellular senescence and inflammatory programs are unintended consequences of CRISPR-Cas9 gene editing in hematopoietic stem and progenitors cells

17h00 **Shengwen Zhang**
LogicBio
OR40: Nuclease-free, promoterless recombinant AAV-mediated genome editing restores function of hepatocytes leading to selective advantage and repopulation in mouse models with liver disease

17h15 **Laura Torella**
CIMA, Pamplona
OR41: In vivo deletion of glycolate oxidase (GO) using double-nicking CRISPR/Cas9 for an efficient and safer treatment of Primary Hyperoxaluria type I (PH1)

17h30 **Felix Lansing**
Dresden University
OR42: Correction of a Factor VIII genomic inversion with designer-recombinases

Parallel 5c: Gene therapy: A bridge to fight infectious diseases

Studio 3
15h40 - 17h45

Chairs: *Emily Turner, BIll and Melinda Gates Foundation*
Jennifer Adair, Fred Hutchinson Cancer Research Center

15h45 **Frank Buchholz**
TU Dresden
INV53: Development of Designer-Recombinases to target retroviral infections

16h15 **Luk Vandenberghe**
Harvard Medical School
INV54: An AAV-based single dose, room-temperature stable COVID-19 vaccine with durable immunogenicity

16h45 **Scott Kitchen**
UCLA
INV55: Engineering CAR-T cells and Cell-Based Vaccines against HIV

17h15 **Alexander Prokofyev**
BIOCAD, St Petersburg
OR43: A novel effective vaccine against SARS-CoV-2 based on recombinant adeno-associated virus serotype 5 (AAV5)

17h32 **Cissy M. Kityo**
Joint Clinical Research Centre, Uganda
OR44: Gene therapy as a means to fight infectious disease and the particular challenges in low and middle income countries

17h45 - 18h10

PROGRAMME 21 OCTOBER 2021

Plenary 5: SARS-Cov2

Studio 1 18h10 - 19h45

Chairs: *Axel Schambach, Hannover Medical School*
Uta Griesenbach, Imperial College London

18h15	**Alexander Douglas** *Jenner Institute, Oxford* **INV57: Collaborative manufacturing of an adenovirus-vectored COVID-19 vaccine: from academia to a billion doses in 18 months**
18h42	**James Miskin** *Oxford Biomedica* **INV58: Delivering the AZ / University of Oxford COVID-19 vaccine – an OXB perspective**
19h15	**Zhengli Shi** *Wuhan Institute of Virology* **INV56: SARS-CoV-2 mutations, pathogenesis, immune escape and its natural reservoirs**

PROGRAMME 22 OCTOBER 2021

08h30 - 08h55 REGISTRATION

Parallel 6a: Oncolytic viruses	Parallel 6b: Stem cells & regenerative medicine	Parallel 6c: Manufacturing
Studio 1 08h55 - 11h00	**Studio 2** 08h55 - 11h00	**Studio 3** 08h55 - 11h00
Chairs: *Ramon Alemany, IDIBELL, Barcelona* *Tomoki Todo, The University of Tokyo*	*Chairs:* *Paula Rio, CIEMAT, Madrid* *Ander Izeta, Biodonostia, San Sebastian*	*Chairs:* *Andy Baker, University of Edinburgh* *Ulrike Köhl, University Hospital, Leipzig*
09h00 **Tomoki Todo** *The University of Tokyo* **INV60: Development and approval of oncolytic herpes virus G47Δ for malignant glioma in Japan**	09h00 **Maurilio Sampaolesi** *KU Leuven* **INV62: 2D and 3D stem cell-based models to reveal new therapeutic targets for muscular dystrophies**	09h00 **Eduard Ayuso** *Dinaqor, Zurich* **INV64: Navigating manufacturing and analytical technologies for AAV vectors**
09h30 **Victor Van Beusechem** *Amsterdam UMC* **INV61: Next-generation oncolytic adenoviruses: preclinical and early clinical evaluation**	09h30 **César Nombela-Arrieta** *University Hospital Zurich* **INV63: Long-term effects of chronic viral infections on the bone marrow microenvironment and hematopoietic stem cell function**	09h30 **Stephen Howe** *GSK, Stevenage* **INV65: Manufacturing process development to industrialise ex vivo gene therapy**
10h00 **Sara Feola** *University of Helsinki* **OR45: Exploiting Pre-existing immunity to enhance oncolytic cancer immunotherapy**	10h01 **Serena Scala** *SR Tiget, Milan* **OR49: Haematopoietic reconstitution dynamics of mobilized peripheral blood- and bone marrow-derived haematopoietic stem/progenitor cells after gene therapy**	10h00 **Lea Krutzke** *University of Ulm* **OR53: Process-related impurities in the ChAdOx1 nCov-19 vaccine**
10h14 **Nina Volf** *ICGEB, Trieste* **OR46: In vivo secretome screening reveals EMID2 as a new anti-invasive molecule preventing metastasis through modulation of tumour microenvironment**	10h15 **Dario Gajewski** *University of Göttngen* **OR50: CRISPR/Cas9-mediated rescue of osteoclast function in a stem cell model of osteopetrosis**	10h15 **Michael Grant** *Achilles Therapeutics* **OR54: Multicentre, prospective research protocol for development of a clonal neoantigen-reactive T cell therapy pipeline across multiple tumour types**
10h31 **Patricia García Rodríguez** *Insituto Carlos III, Madrid* **OR47: MAVS-/- mesenchymal stem cells as oncolytic adenovirus ICOVIR-5 carriers for cancer treatment**	10h31 **Marco Luciani** *SR Tiget, Milan* **OR51: Transcriptional and epigenetic identity of hiPSC-derived neural stem/progenitor cells: Implications for cell therapy approaches**	10h30 **Michael Magnussen** *University College London* **OR56: Modelling cardiogenesis using intravital hydrogel bioprinting and tissue engineering identifies novel roles for hyaluronan and ECM mechanics during human trabeculogenesis**
10h45 **Shifaa Abdin** *Rebirth, Hannover Medical School* **OR48: A universal platform to generate chimeric antigen receptor macrophages from human stem cell sources**	10h45 **Natalyia Basalova** *Moscow State University* **OR52: Extracellular vesicles produced by mesenchymal stromal cells inhibit rather the progression than initiation of fibrosis**	10h45 **Michelle Leblanc** *Generation Bio* **OR55: Development of a novel lipid nanoparticle with widespread photoreceptor delivery of ceDNA & mRNA cargos**

11h00 - 11h25 BREAK

Parallel 7a: Safety Studio 1 11h25 - 13h31 Chairs: Christof von Kalle, Charité Hospital, Berlin; Zoltan Ivics, Paul Elrich Institute, Langen	Parallel 7b: Liver & metabolic diseases II Studio 2 11h25 - 13h33 Chairs: Fätima Bosch, UAB Barcelona; Alessio Cantore, SR Tiget, Milan	Parallel 7c: CNS & sensory III Studio 3 11h25 - 13h30 Chairs: Aurora Pujol Onofre, IDIBELL, Barcelona; Françoise Piguet, Insitut du Cerveau et de la Moëlle
11h30 **Axel Schambach** *Hannover Medical School* **INV66: Towards tailored and function-controlled gene therapies**	11h30 **Laura Sepp-Lorenzino** *Intellia Therapeutics* **INV68: Advances in CRISPR/Cas9 Therapeutic Genome Editing for In Vivo and Ex Vivo Applications**	11h30 **Jing Nie** *Indiana University School of Medicine* **INV70: Modeling CHARGE syndrome in human inner ear organoids**
12h00 **Ian Alexander** *The University of Sydney* **INV67: AAV vector safety considerations in liver-targeted gene therapy**	12h00 **Thierry VandenDriessche** *VUB, Brussels* **INV69: Next-generation gene therapies for haemophilia: moving beyond FIX-Padua**	12h00 **Scott Ellis** *Gyroscope Therapeutics* **INV71: GT005, an investigational AAV2 vector encoding complement factor I, for geographic atrophy**
12h31 **David Williams** *Harvard Medical School* **OR57: Integration site analysis in patients with cerebral adrenoleukodystrophy (CALD) treated with elivaldogene autotemcel (Lenti-D; eli-cel) gene therapy**	12h30 **Nicholas Weber** *Vivet* **OR61: Durable efficacy of AAV-based gene therapy for PFIC3 (VTX-803) in mice at different disease stages**	12h30 **Riccardo Privolizzi** *University College London* **OR66: In vivo evaluation of novel synthetic promoters for CNS gene therapy**
12h46 **Daniela Cesana** *SR Tiget, Milan* **OR58: Monitoring of vector integration sites in in vivo gene therapy approaches by Liquid-Biopsy-Integration-Site-Sequencing**	12h45 **Francesco Starinieri** *SR Tiget, Milan* **OR62: Administration during liver growth improves the efficiency of lentiviral vector based gene therapy in mice**	12h45 **Ralf Schmid** *University of Pennsylvania* **OR67: Developing a gene-replacement therapy for CDKL5-deficiency disorder**
13h02 **Randy Chandler** *NIH, Bethesda, MD* **OR59: Sequencing of genotoxic recombinant adeno-associated viral integrations events found in murine hepatocellular carcinomas reveals conservation of specific transgene elements**	13h01 **Louisa Jauze** *Genethon, Evry* **OR63: Hepatocyte-directed AAV gene transfer drives efficient long-term rescue of the metabolic impairment in a mouse model of glycogen storage disease type Ia**	13h00 **Patrizia Tornabene** *Tigem, Naples* **OR68: Therapeutic AAV-mediated homology-independent targeted integration in the retina**
13h15 **Mariana Loperfido** *AvroBio* **OR60: High-resolution cellular and molecular follow-up of lysosomal disorder patients treated with hematopoietic stem cell lentiviral gene therapy**	13h17 **Mark Thomas** *Royal Perth Hospital* **OR64: AVR-RD-01, an investigational lentiviral gene therapy for Fabry disease: Interim results from Phase 1 and Phase 2 studies**	13h15 **Sara Marco Costa** *CBATEG, UAB, Barcelona* **OR69: AAV-mediated gene therapy to treat Niemann-Pick Type C2**
13h31 - 13h55 BREAK		

Parallel 8a: Hematopoietic & bleeding disorders II

Studio 1
13h55 - 16h01

Chairs: *Axel Schambach, Hannover Medical School*
Marina Cavazzana, Institut Imagine, Paris

14h00 **David Williams**
Harvard Medical School, MA
INV72: Clinical application of post-transcriptional silencing of BCL11A

14h31 **Marina Cavazzana**
Institut Imagine, Hoptial Necker, Paris
INV73: Gene Therapy in genetic disorders

14h57 **José Luis Lopez Lorenzo**
University Hospital Jimenez Diaz, Madrid
OR70: Lentiviral mediated gene therapy for pyruvate kinase deficiency: Interim results of a global phase 1 study for adult and pediatric patients

15h12 **Laura Ugalde Díaz**
Ciemat, Madrid
OR71: Homology independent gene editing strategies to correct hematopoietic stem cells from Fanconi Anemia A patients

15h29 **Chi Yuan Zhang**
Dana Faber Cancer Institute, Boston
OR72: A novel DNA oligo-based repair strategy for the functional correction of Shwachman-Diamond Syndrome

15h45 **Martina Fiumara**
SR Tiget, Milan
OR73: Assessing stealth and sensed base editing in human hematopoietic stem/progenitor cells

Parallel 8b: AAV immunology

Studio 2
13h55 - 16h00

Spark THERAPEUTICS

Chairs: *Hildegard Büning, Hannover Medical School*
Anne Galy, Genethon, Evry

14h00 **Ying Kai Chan**
Harvard University
INV75: New applications, new immunological challenges

14h30 **Giuseppe Ronzetti**
Genethon, Evry
INV76: Overcoming anti-AAV pre-existing immunity to achieve safe and efficient gene transfer in clinical settings

15h00 **George Buchlis**
University of Pennsylvania
OR74: Administering high-dose AAV9 vector in wild-type mice leads to complement activation, liver deposition, and concomitant liver-transaminase elevations

15h15 **Gwladys Gernoux**
University of Nantes
OR75: Prevalence study of cellular capsid-specific immune response to AAV9 reveals an unconventional T cell immunity

15h30 **Malo Journou**
University of Nantes
OR76: Intramuscular delivery of rAAV in non-human primates results in a chronic local inflammation in situ without the loss of transgene expression

15h45 **Lorenzo D'Antiga**
Hospital Pap Giovanni XXIII, Bergamo
OR77: Adeno-associated virus vector mediated gene therapy for Crigler Najjar syndrome: Results of safety and efficacy from the dose escalation phase of the CareCN clinical trial

Parallel 8c: Cutting edge gene and cell therapy research in America

Studio 3
13h55 - 16h00

ASGCT American Society of Gene + Cell Therapy

Chairs: *Stephen Russell, Mayo Clinic, Rochester, MA*
Beverly Davidson, ASGCT President, Children's Hospital of Philadelphia

13h55 **Stephen Russell**
Mayo Clinic, Rochester
Welcome

14h10 **Beverly Davidson**
Children's Hospital of Philadelphia
INV77: Advances in regulating gene therapies

14h42 **Hans-Pieter Kiem**
Fred Hutchinson Cancer Research Center
INV78: Engineering hematopoietic stem cells for gene and immunotherapy

15h27 **Jeffrey Chamberlain**
University of Washington
INV79: AAV-mediated muscle transduction of micro- and mini-dystrophins

16h01 - 16h25 BREAK

Plenary 6: Gene editing

Intellia THERAPEUTICS

Studio 1 16h25 - 18h30

Chairs: *Hildegard Büning, Hannover Medical School*
Luigi Naldini, SR TIget, Milan

16h30 **Dan Bauer**
Harvard University, MA
INV81: Gene editing for blood disorders

17h00 ESGCT Young Investigator Award
Julian Grünewald
Massachusetts General Hospital
INV82: Engineering reduced size CRISPR prime editor proteins that retain efficient activities in human cells

17h30 **Toni Cathomen**
University of Freiburg
INV83: Assessing and mitigating off-target effects in therapeutic genome editing

18h00 **Sam Sternberg**
Columbia University, NYC
INV84: Targeted DNA integration without double-strand breaks using CRISPR RNA-guided transposons