Société Française de Thérapie Cellulaire et Génique
SFTCG

SFTCG Annual Meeting
16-18 March 2022
Ecole normale supérieure de Lyon

ciri Centre International de Recherche en Infectiologie
ENS DE LYON

Programme

Wednesday 16 March

13:00-14:00	**Registration**
14:00-14:45	**Opening Keynote** Chair: Els Verhoeyen **INV01** **Nathalie Cartier,** *BrainVectis, AskBio, Paris* Gene therapy for CNS
14:45-16:15	**Session 1: Gene editing in gene therapy** Chairs: François-Loïc Cosset, François Moreau-Gaudry **INV02** **Fernando Larcher**, *Ciemat / Ciberer, Madrid* CRISPR/Cas9-based gene editing strategies for clinically-relevant ex vivo correction of Recessive Dystrophic Epidermolysis Bullosa **INV03** **Annarita Miccio**, *Institut IMAGINE, Paris* CRISPR/Cas9-based therapeutic strategies for β-hemoglobinopathies **OR01** **Victor Tiroille**, *Université Côte d'Azur, INSERM, C3M, 06204 Nice, France* Nanoblades allow high-level genome editing in organoids **OR02** **Juliette Rosier**, *Université Bordeaux* CRISPR-Cas9 globin editing can induce megabase-scale copy-neutral losses of heterozygosityin hematopoietic cells leading to imprinting defects
16:15-16:45	**Coffee break**
16:45-18:45	**Session 2: In vivo gene therapy** Chairs: Eduard Ayuso, Capucine Trollet GENETHON CURE THROUGH INNOVATION **INV04** **Gloria Gonzalez-Aseguinolaza**, *CIMA, Pamplona* Gene therapy for liver inherited diseases **INV05** **Martina Marinello**, *Genethon, Evry* Gene therapy of spinal muscular atrophy by single-stranded AAV9 vectors **INV06** **Frederic Thalheimer**, *Paul-Ehrlich-Institut, Langen* Receptor targeted viral vectors for *In vivo* delivery **OR03** **Celia Sourd**, *Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE* An AAVpo1A1 vector expressing MTM1 corrects skeletal muscle pathology with detargeted liver transduction in myotubular myopathy mice **OR04** **Mathieu Mevel**, *Nantes Université, TaRGeT - Translational Research in Gene Therapy, INSERM UMR 1089, CHU de Nantes* Chemically tyrosine modified AAV vectors: an innovative technological platform to boost gene delivery
18:45-19:30	**SFTCG General Assembly**
19:15	**Welcome reception and Posters**

Thursday 17 March

08:30-09:00 **Registration**

09:00-11:00 **Session 3: Innovative approaches**
Chairs: Michel Pucéat, Els Verhoeyen

INV07 **Denis Furling,** *Centre de Recherche en Myologie, UMRS 974, Paris*
Decoy gene therapy for Myotonic Dystrophy

INV08 **Eduard Ayuso,** *Dinaqor, Zurich*
A genetic medicine platform for the treatment of severe inherited cardiac diseases

INV09 **Valérie Dardalhon,** *IGMM, Montpellier*
Targeting the metabolic environment to modulate CAR-T cell effector function

OR05 **Audrey Page,** *CIRI; Inserm U1111*
A new synthetic circuit for B cell reprogramming to cure cancer

OR06 **Tina Briolay,** *Université de Nantes*
Development of a bio-inspired nanovector for targeted cancer gene therapy

11:00-11:30 **Coffee break**

11:30-13:30 **Session 4: Cancer gene therapy**
Chairs: Nicolas Boisgerault, Pierre Cordelier

INV10 **Axel Schambach**, *Hannover Medical School*
From CARs to TRUCKs: Towards next-generation immunotherapeutics

INV11 **Hélène Nègre**, *Servier*
T2EVOLVE: Standardization of preclinical and clinical development of engineered T-cell therapy in Europe—a cross-functional multi-stakeholder initiative

INV12 **Els Verhoeyen**, *CIRI, ENS de Lyon and C3M, Nice*; **Alejandra Gutierrez-Guerrero**, *CIRI, ENS de Lyon*
Novel lentiviral pseudotypes for T and NK cell gene therapy and 'nanoblades' for efficient gene editing in hematopoietic gene therapy target cells.

OR07 **Adrien Krug**, *Université Côte d'Azur/C3M*
CD8-targeted lentiviral vectors to generate CAR T cells in vivo as treatment for T cell lymphoma

OR08 **Ugo Hirigoyen**, *Nantes Université, Inserm UMR 1307, CNRS UMR 6075, Université d'Angers, CRCI2NA*
Characterization of tumor extracellular vesicles produced during oncolytic infection

13:30-15:00 **Lunch and Posters**

15:00-16:45 **Session 5 : Stem Cells and iPS**
Chairs: John De Vos, Leila Maouche-Chrétien

INV13 **Guillaume Rousseau**, *EryPharm, Paris*
Industrial production of cultured red blood cells: a model for future large-scale cell therapies finally within reach

INV14 **Pierre Savatier,** *Stem Cell and Brain Research Institute, Lyon*
Interspecies systemic chimeras: a new path for exploring pluripotent stem cell biology and modeling human early development

INV15 **Christelle Monville,** *Istem, Evry*
Retinal pigment epithelial cells derived from human embryonic stem cells disposed on human amniotic membrane: An update on the french RP clinical trial

	OR09 **Carine Bourdais,** *IRMB, Univ Montpellier, INSERM, CHU Montpellier* Combined cellular and gene therapy to treat primary ciliary dyskinesia
16:45-17:15	**Coffee break**
17:15-19:15	**Session 6: Towards clinical trials** Chairs: Anne Galy, Françoise Piguet **INV16** **Françoise Piguet**, *Inserm, Paris* Gene therapy of Amyotrophic Lateral Sclerosis **INV17** **Olivier Nègre**, *GCTi* Gene and cell therapies: G&CTI, a think tank to face the multiple challenges **INV18** **Thomas Roujeau**, *Hôtpital Gui de Chauliac, Montpellier* Intracerebral gene therapy in 2 patients with aromatic-L acid decarboxylase (AADC) deficiency **OR11** **Miryam Mebarki**, *Unité de Thérapie Cellulaire, Hôpital Saint-Louis, AP-HP* Development of a human umbilical cord-derived mesenchymal stromal cell (UC-MSC)-based advanced therapy medicinal product (ATMP) to treat immune and/or inflammatory diseases **OR12** **Barbara Garmy-Susini**, *Inserm UMR1048* 15-Lipoxygenase drives inflammation resolution and Treg trafficking in lymphedema

Friday 18 March

09:00-09:30	**Registration**
09:30-11:00	**Session 7: Gene therapy & more** Chairs: Olivier Nègre, Juan Bueren Pfizer Maladies Rares **INV19** **Anne Galy,** *Genethon, Evry* Gene therapy using lentiviral vectors : current clinical results and novel perspectives **OR13** **Elena-Gaia Banchi,** *Institut du Cerveau et de la Moelle épiniere* Gene Therapy For Spinocerebellar Ataxia 7 : Restoring Cholesterol Metabolism **OR14** **Juliette Lemoine,** *Genethon, UMR_S951, Inserm, Univ Evry, Université Paris Saclay, EPHE* Novel AAV capsid variant for muscle-directed gene therapy
11:00-11:30	**Coffee break**
11:30-12:30	**Closing keynote** Chairs: Els Verhoeyen **INV21** **Juan Bueren**, *CIEMAT/Ciberer, Madrid* Gene therapy in hematopoietic stem cell diseases: The Fanconi anemia model **Poster and oral presentation awards**

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