

# Annual meeting of SFTCG

## ENS, Lyon, France

### 11 to 13 March, 2020

#### Wednesday 11 March

09:00 **Matinée portes ouvertes pour les lycéens de Lyon** Speed-searching:  
11:00 Interaction avec les chercheurs et leurs outils de travail

#### **Session pour les patients et associations de patients**

- 1) Introduction générale sur la thérapie génique
- 2) Atelier individuel sur différents thèmes
- 3) Table Rond introduit par un film

**13:30 Registration**

**14:00 Opening Keynote**

Chair: François-Loïc Cosset, Els Verhoeyen

**INV01 Fulvio Mavilio**, *Audentes Therapeutics, San Francisco*  
TBC

**14:45 Session 1 : Gene editing in gene therapy**

**15:45** Chair: François-Loïc Cosset, François Moreau-Gaudry

**INV02 Claudio Mussolino**, *Freiburg University*

Advances in precision genome and epigenome editing for human gene therapy

**INV03 Fernando Larcher**, *Ciemat / Ciberer, Madrid*

CRISPR/Cas9-based gene editing strategies for clinically-relevant ex vivo correction of Recessive Dystrophic Epidermolysis Bullosa

**INV04 Annarita Miccio**, *Imagen, Paris*

CRISPR/Cas9-based therapeutic strategies for  $\beta$ -hemoglobinopathies

2 selected abstracts

15:45

Coffee break

16:15

16:15 **Session 2 : *In vivo* gene therapy**

Chair: Eduard Ayuso, Capucine Trollet

18:15

**INV05 Gloria Aseguinolaza**, *CIMA, Pamplona*

Gene therapy for inherited liver diseases

**INV06 Martina Marinello**, *Geneton, Evry*

Gene therapy of spinal muscular atrophy by single-stranded AAV9 vectors

**INV07 Fred Thalheimer**, *Paul Elrich Institute, Langen*

TBC

2 selected abstracts

## SFTCG General Assembly

18:30 Welcome reception and Posters

### Thursday 12 March

9:00 **Session 3: Innovative approaches**

Chairs: Michel Pucéat, Els Verhoeyen

11:00

**INV11 Tuan Nguyen**, *Golliver Therapeutics, Nantes*

Pluripotent stem cell-based ATMPs for liver repair

**INV12 Jens Schwamborn**, *University of Luxemburg*

Human midbrain organoids for Parkinson`s disease *in vitro* modeling and drug development in

**INV13 Valérie Dardalhon**, *IGMM, Montpellier*

Targeting the metabolic environment to modulate CAR-T cell effector function

2 selected abstracts

11:00

Coffee Break

11:30

11:30 **Session 4: Cancer gene therapy**  
Chair: Nicolas Boisgerault, Pierre Cordelier

13:30 **INV14 Andrea Biondi**, *University of Milano Bicocca, Monza*  
TBC

**INV15 Nicolas Ferry**, *Servier*

CAR-T cells, the slow road from autologous to allogenic origin

**INV16 Yaohe Wang**, *Barts Cancer Institute, London*

TBC

2 selected abstracts

13:30

**Lunch and Posters**

15:00

15:00 **Session 5 : Stem Cells and iPS**

Chair: John De Vos, Leila Maouche-Chrétien

17:00

**INV08 Guillaume Rousseau**, *EryPharm, Paris*

Ex vivo production of Red Blood Cells from stem cells for transfusion purposes

**INV09 Pierre Savatier**, *Stem Cell and Brain Research Institute, Lyon*

Naive-state pluripotency and chimeric competency in human and non-human primates

**INV10 Christelle Monville**, *Istem, Evry*

TBC

2 selected abstracts

17:00

**Coffee Break**

17:30

17:30 **Session 6: Towards clinical trials**

Chair: Anne Galy, Nathalie Cartier

19:30

**INV17 Françoise Piguet**, *Inserm, Paris*

Restoring neuronal cholesterol metabolism in ALS mouse model

**INV18 Matthias Titeux**, *Institut Imagine, Paris*

TBC

**INV19 Magda Papadaki**, *Merck, London*

Understanding and managing regulatory and reimbursement uncertainty during ATMP development: commercial and business impacts

2 selected abstracts

20:00 **Congress dinner and party**

## Friday 13 March

09:30 **Session 7: Clinical trials & more**

11:30 Chair: Olivier Nègre, Federico Mingozi

**INV20 Christian Jorgensen, IRMB, Montpellier**

TBC

**INV21 Michael Hocquemiller, Lysogene**

LYS-SAF302 gene therapy study in mucopolysaccharidosis type IIIA (MPS IIIA) children

**INV22 Francisco Fernandez Aviles, Hospital General Universitario Gregorio Marañón, Madrid**

Cell therapies for cardiac repair: A mechanistic approach

2 selected abstracts

**11:30**

**Coffee break**

**12:00**

12:00 **Closing keynote**

13:00 Chair: Els Verhoeyen

**INV23 Juan Bueren, CIEMAT/Ciberer, Madrid**

Gene Therapy in Hematopoietic Stem Cell Diseases: The Fanconi anemia model

Poster and oral presentation awards