

# ESGCT SPRING SCHOOL 2023

28-31 March 2023  
Giens, France

## Tuesday 28 March

<b>Registration</b> 15:00-16:30		
<b>Workshops (40 students per workshop)</b> 16:30-19:00		
<b>Workshop 1: Form your own opinion – how to judge a scientific publication</b> 16:30-19:00 Hildegard Büning, <i>ESGCT Education &amp; Training</i> Els Verhoeyen, <i>President, SFTCG, Board Member, ESGCT</i>	<b>Workshop 2: How to write a scientific manuscript</b> 16:30-19:00 Leszek Lisowski, <i>University of Sydney</i> Thomas Gallagher, <i>Human Gene Therapy</i>	<b>Workshop 3: Ethical consideration in experimental, pre-clinical and clinical gene therapy</b> 16:30-19:00 Frank Staal, <i>University of Leiden</i>
<b>Dinner</b> 19:30		

## Wednesday 29 March

<b>Welcome Address</b> 08:30-09:00 Juan Bueren, <i>President, ESGCT</i> Els Verhoeyen, <i>President, SFTCG; Board Member, ESGCT</i> Hildegard Büning, <i>ESGCT Education &amp; Training</i>
<b>Keynote</b> 09:00-10:00 Juan Bueren, <i>Ciemat Madrid</i> INV01: Moving from Concept to Clinical Reality – 33 years from the first human clinical trial to now



### Session 1: Gene therapy tools - insights by experts (LV)

10:00-11:20

**Axel Schambach**, *Hannover Medical School*

**INV02:** What you should know about retro/lentiviral vector design

**Els Verhoeven**, *University of Nice*

**INV03:** How to improve nature – the next generation of lentiviral vectors

### Coffee Break

11:20-11:50

### Keynote

11:50-12:50

**Frank Staal**, *Leiden University*

**INV04:** Developing stem cell based gene therapy for Recombinase deficiencies

### Lunch

12:50-13:50

### Team building activity

13:50-15:00

### Session 2: Gene therapy tools - insights by experts (AAV)

15:00-16:20

**Hildegard Büning**, *Hannover Medical School*

**INV05:** Almost a virus – the adeno-associated virus (AAV) vector system

**Leszek Lesowski**, *University of Sydney*

**INV06:** How to improve nature – the next generation of AAV vectors

### Keynote

16:20-17:20

**Gloria Gonzalez-Aseguinolaza**, *FIMA Pamplona*

**INV07:** Liver-directed Gene Therapy – the long journey to the market

### Coffee Break

17:20-17:50

### Session 3: Gene therapy tools - insights by experts (non-viral vectors)

17:50-19:10

**Pieter Vader**, *University Medical Center Utrecht*

**INV08:** Extracellular vesicles

**Tristan Montier**, *University of Brest*

**INV09:** Extracellular vesicles for targeted nucleic acid delivery: Problem solved?



## Dinner

19:30

20:30 After dinner keynote

Nathalie Cartier, *AskBio, Paris*

INV010: Gene Therapy for neurodegenerative diseases from genetic to complex diseases: delivery and targets

## Thursday 30 March

### Keynote

09:00-10:00

Toni Cathomen, *University Hospital Freiburg*

INV11: Risk assessment of gene editing associated off-target effects

### Session 4: Gene Therapy 2.0 – The Age of precision Gene Therapy

10:00-11:20

Paula Rio, *Ciemat Madrid*

INV12: Gene editing is coming to life as treatment strategy in Fanconi anemia

Matthias Titeux, *Institut Imagine, Paris*

INV13: Gene editing strategies for rare skin disorders

### Coffee Break

11:20-11:50

### Keynote

11:50 -12:50

Anne Galy, *Genethon, Evry*

INV14: The immune system does not like it to be tricked

### Lunch

12:50-13:50

### Team building activity

13:50-15:00

### Session 5: The payload

15:00-16:20

Zoltan Ivics, *Paul Ehrlich Institute, Langen*

INV15: Wide awake and ready to move: non-viral genome engineering with Sleeping Beauty transposon vectors

Ivana Trapani, *Tigem Naples*

INV14: How to deliver large genomes in a too small vehicle

### Keynote

16:20 -17:20

Vincenzo Cerullo, *University of Helsinki*

INV16: Dressing viruses in tumor's clothing – new strategies in anti-tumor therapy



### Coffee Break

17:20-17:50

### Session 6: Fighting cancer with novel weapons

17:50-19:10

**Andrea Schmidts**, *Technical University of Munich*

**INV17:** CAR-T cells – how to make a living drug

**Eliana Ruggiero**, *HSR, Milan*

**INV18:** The world beyond CAR T cells

### Dinner

19:30

## Friday 31 March

### Keynote

09.00-10.00

**Alberto Auricchio**, *Tigem Naples*

**INV19:** Vision – Gene Therapy for fighting blindness

### Coffee Break

10.00-10.30

### Session 7: Pre-clinical and clinical gene therapy

10:30-11.50

**Giuliana Ferrari**, *SR Tiget, Milan*

**INV20:** Gene therapy for hemoglobinopathies: past, present and future

**Alessandro Aiuti**, *HSR Tiget, Milan*

**INV21:** Gene Therapy for primary immunodeficiencies, the prime target for lentiviral vector ex vivo gene therapy, is reaching its next level

### Keynote

11:50-12.50

**Claire Booth**, *University College London*

**INV22:** It is all about accessibility

### Closing

12:50

**Juan Bueren**, *President, ESGCT*

**Els Verhoeven**, *President, SFTCG; Board Member, ESGCT*

**Hildegard Büning**, *ESGCT Education & Training*