

# 2024 Workshop on Gene Editing & Cell Therapy



Nantes | 21 & 22 March 2024

## PROGRAMME

### Thursday 21 March: SFTCG Academy

12:00-13:00	<b>Registration and light lunch</b>
13:00-14:00	<p><b>Mentoring</b></p> <p>Chair: <b>Els Verhoeyen</b>, <i>University of Nice</i></p> <p><b>INV01 Zoltan Ivics</b>, <i>Paul Ehrlich Institute, Langen</i> Digging in the Dirt: How Archeogenetics and Synthetic Biology Impacted Gene Therapy</p>
14:00-15:30	<p><b>Oral presentations from students</b> (10 minute presentation and 20 minutes for questions and mentoring)</p> <p>Chairs: <b>Sophie Gomez</b>, <i>CRCL, Lyon</i>; <b>Capucine Trollet</b>, <i>Sorbonne University - Institut de Myologie</i>; <b>Els Verhoeyen</b>, <i>University of Nice</i></p> <p><b>OR01 Clémence Lièvre</b>, <i>TaRGeT, Nantes</i> Skeletal muscle organoids for preclinical gene therapy with recombinant AAV vectors</p> <p><b>OR02 Clément Morival</b>, <i>TaRGeT, Nantes</i> Modelling Stargardt disease using three-dimensional retinal organoids</p> <p><b>OR03 Carine Bourdais</b>, <i>IRBM, Montpellier</i> Combined cellular and gene therapy to treat primary ciliary dyskinesia</p>
15:30-16:00	<b>Coffee Break</b>
16:00-18:30	<p><b>Oral presentations from students</b> (10 minute presentation and 20 minutes for questions and mentoring)</p> <p>Chairs: <b>Olivier Nègre</b>, <i>Smart Immune, Paris</i>; <b>Françoise Piguet</b>, <i>TIDU GENOV, ICM, Paris</i></p> <p><b>OR05 Manon Lucas</b>, <i>TaRGeT, Nantes</i> High systemic dosing of AAV9 vectors: a relevant rat model to study the impact of vector immunogenicity in the liver</p> <p><b>OR06 Laurie Lacombe</b>, <i>Généthon Evry</i> NHEJ inhibition increases homology-mediated AAV integration in hematopoietic cells</p> <p><b>OR07 Jean Baptiste Ducloyer</b>, <i>TaRGeT, Nantes</i> Study of retinal microglial cells after sub-retinal injection of recombinant AAV in pigs</p> <p><b>OR08 Marie Esnard</b>, <i>GEPEA – St Nazaire / TaRGeT, Nantes</i> Development of an AAV bioproduction technological platform in microalgae</p>

## Friday 22 March: Theme Day

Thematic morning on gene editing	
08:00-09:00	<b>Registration</b>
09:00-10:30	<p><b>Session 1</b> (10 minute presentation and 5 minutes for questions)</p> <p>Chairs: <b>François Moreau Gaudry</b>, <i>Equipe Biothérapie INSERM U1035, Université Bordeaux</i>; <b>Caroline Le Guiner</b>, <i>Nantes Université, CHU de Nantes, INSERM TaRGeT Lab</i></p> <p><b>INV02 Carine Giovannangeli</b>, <i>Département AVIV, MNHN, CNRS UMR7196, INSERM U1154, Paris</i></p> <p><b>INV03 Annarita Miccio</b>, <i>Institut Imagine</i> Genome editing approaches for hematopoietic disorders</p> <p><b>OR09 Paloma Navas Navarro</b>, <i>Généthon Evry</i> Treatment of myotonic dystrophy type 1 with CRISPR/Cas9</p> <p><b>OR10 Margaux Melka</b>, <i>Institut des Neurosciences de Grenoble</i> Mutation independent CRISPR/Cas9-induced allele deletion results in vitro in a functional benefit for dominant RYR1 mutation</p> 
10:30-11:00	<b>Coffee Break</b>
11:00-12:30	<p><b>Session 2</b> (10 minute presentation and 5 minutes for questions)</p> <p>Chairs: <b>Mario Amendola</b>, <i>INTEGRARE UMR951, Genethon, Evry</i>; <b>Matthias Titeux</b>, <i>Institut Imagine, Paris</i></p> <p><b>INV04 Maria Silvia Roman Azcona</b>, <i>University of Freiburg</i> Epigenome editing of immune checkpoints in CAR T cells</p> <p><b>INV05 François Moreau Gaudry</b>, <i>University of Bordeaux</i> Genotoxicity risk induced by CRISPR-Cas9: understanding and preventing it</p> <p><b>OR11 Juliette Varin</b>, <i>TaRGeT, Nantes</i> Homology-independent targeted integration (HITI) as possible treatment for Stargardt disease</p> <p><b>OR12 Hugo Malki</b>, <i>Institut de la Vision, Paris</i> Delivery of Cas9 ribonucleoprotein to the retina of mice with Retinitis pigmentosa</p> 
12:30-14:00	<b>Lunch</b>
Thematic afternoon on cell therapy	
14:00-15:30	<p><b>Session 3</b> (10 minute presentation and 5 minutes for questions)</p> <p>Chairs: <b>Christelle Monville</b>, <i>INSERM/UEVE UMR 861, I-STEM, AFM, Corbeil-Essonnes</i>; <b>Leila Maouche Chrétien</b>, <i>INSERM U 1163 / CNRS ERL 8254, Institut IMAGINE</i></p> <p><b>INV08 Philippe Menasché</b>, <i>Université Paris Cité</i> Cell therapy for cardiac diseases: successes and remaining challenges</p> <p><b>INV09 Karl Rouger</b>, <i>PanTher - INRAe, ONIRIS, UMR 703 – Nantes</i> Muscle repair potential of adult stem cells through different modes of action</p> <p><b>OR13 Isabelle Prost</b>, <i>Promega, France</i> New technologies for CAR-T characterization and potency testing</p> <p><b>OR14 Allwyn Pereira</b>, <i>TaRGeT, Nantes</i> Multiomic analyses reveals epigenome remodelling and transcriptional co-factors as critical determinants for neuronal cell therapy</p>
15:30-16:00	<b>Coffee Break</b>

<p><b>16:00-17:30</b></p>	<p><b>Session 4</b> (10 minute presentation and 5 minutes for questions) Chairs: <b>Oumeya Adjali</b>, <i>TaRGeT - INSERM UMR 1089, IRS 2 - Nantes Biotech</i>; <b>Emmanuel Payen</b>, <i>CEA, Fontenay aux Roses</i> <b>INV11 Jérémie Martinet</b>, <i>Rouen University</i> Irradiation as an adjuvant for CAR-T cell therapy <b>INV12 Vasco Meneghini</b>, <i>Hospital San Raffaele, Milano</i> Tackling neurodegeneration with neural stem and progenitor cells <b>OR15 Camille Le Guen</b>, <i>CHU Nantes</i> Can academic structures improve access to CAR-T cells?</p>
<p><b>17:30</b></p>	<p><b>Closing</b></p>

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