Under the High Patronage of Mr Emmanuel Macron President of the French Republic









HYBRID : Next generations of gene & cell therapies

8&9 February 2024

Day 1: 8 February 2024

8:00am Welcome coffee 8:45am Official speech

Bruno Bonnell, General Secretary for Investment – France 2030 (SGPI) 8:55am Opening ceremony Thomas Lombes, Deputy CEO for strategy, Inserm, Thierry Hulot, Chairman of Leem, Marc Bonneville, Chairman of Ariis, Rosalie Maurisse, Head of Health - Innovation Department at Bpifrance

Session 1: 9:15am - 12:45pm

The challenges associated with future viral vectors and genome editing approaches

From the main challenges, avenues and prospects for new vectors to optimized delivery and safety, and tomorrow's genome-editing strategies

Overview: 9:15am

> Oumeya Adjali, Inserm - Gene therapy using AAV viral vectors ; from vector engineering to preclinical evaluation

9:35am Short talks:

- Ana Buj-Bello, Inserm Next generation AAV vectors for gene therapy of muscle disorders
- Alexis Duvergé, CNRS Specific Targeting of Cancer Cells by Lentiviral Vectors
- Maria Grazia Biferi, Spark Therapeutics Targeting CNS with AAV in a large animal
- 10:10 am Questions & Answers 10:30 am Coffee break

11:05 am Short talks:

- Annarita Miccio, Inserm Genome editing approaches for beta-hemoglobinopathies
- Aurélie Bedel, Bordeaux University How to secure CRISPR-Cas9 use?
- Stéphane Boissel, Sparing Vision Pioneering Genomics to Save Sight
- 11:40am Ouestions & Answers
- 11:55am Round table:
 - Sitra Tauscher-Wisniewski, Novartis
 - Anne Douar, Vivet Therapeutics
 - Annarita Miccio, Inserm
- 12:45pm Lunch break

Session 2: 3:00pm – 5:00pm New nucleic acid therapies : applications, vectorization

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ACTIS Attace

From prophylactic to therapeutic vaccines and the various potential uses of nucleic acids. Challenges and barriers associated with the uses of nucleic acids: shelf life, route of administration, targeting, etc.

3:00pm Overview:

• **Chantal Pichon**, Orléans University - The game changing potential of mRNA-based vaccines and therapeutics

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Inserm

- **Pierre Wils**, Sanofi From Innovation to Industrialization: Challenges in Research and Development of mRNA Vaccines
- **Nathalie Mignet**, CNRS Production of lipid nanoparticles formulations for gene and cell therapy
 - **Philippe Barthélémy**, Bordeaux University Therapeutic oligonucleotides: efficient and specific tools to combat diseases
- 4:00pm Questions & Answers

4:15pm Round table:

- Paul Nioi, Alnylam
- Chantal Pichon, Inserm
- Anette Sommer, Pfizer
- Hélène Tran, Servier
- Pierre Wils, Sanofi

B2B Meetings: 5:00pm - 6:30pm

Cocktail reception: 6:30pm – 8:00pm

H B R I D H B R I D

8:30am







Day 2: 9 February 2024

8:00am Welcome coffee

Keynotes **Cécile Martinat** & **Christophe Junot** - National research program in biotherapies and bioproduction

Session 3: 8:45am - 12:00pm Immuno cell based therapies

How can the uses of CAR be extended beyond current applications? How can we improve delivery to solid tumours, the role of the tumour environment, survival, etc.? Applications beyond cancer.

8:45am	 Tamas Shisha, Novartis - An Open-label, Multicenter, Phase 1/2 Study to Assess Safety, Efficacy and Cellular Kinetics of YTB323, a CAR-T Cell Therapy Targeting CD19 for Severe Refractory SLE (Preliminary Results)
9:05am	 Short talks: Mitra Suman, Inserm - Optimizing Adoptive Cellular Therapies in Physio-Chemical Tumor Environments for Superior Anti-Cancer Efficacy without systemic toxicity Emmanuel Donnadieu, CNRS - Predicting efficacy and toxicity of CAR T cells using an ex vivo human model Marie-Caroline Dieu-Nosjean, Inserm - Manipulation of tertiary lymphoid structures as the third generation of immunotherapy Georges Lourenço, Mnemo Therapeutics - A new class of tumor targets for next-generation cancer immunotherapies
9:50am 10:15am	Questions & Answers Coffee break
10:50am	 Round table: Alessandro Crotta, BMS Viggo VanTendeloo, J&J Sante Cundari, Kite- a Gilead company Karine Rossignol, SmartImmune Emmanuel Donnadieu, CNRS
11:40am	Keynote:Lise Alter, Head of AIS - The missions of the Health Innovation Agency
12:00pm	Lunch break

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Session 4: 2:00pm - 4:30pm Cell therapies and beyond

From the various therapeutic applications of cell therapies to exosomes: how to develop clinical uses of cell therapies, address characterisation issues, etc.? How to take full advantage of the coupling between therapeutic effect and vectorisation properties for exoxomes.

2:00pm Short talks:

- Louis Casteilla, Toulouse University MSC-based ATMP: present and future
- **Christelle Monville**, Evry University A stem cell therapy pipeline with focus on retinitis pigmentosa and other rare indications with high unmet medical needs
- Frédéric Desdouits, Treefrog Therapeutics Unlocking cell therapy for all
- **Christian Jorgensen**, CHU of Montpellier Next generation cell therapies for osteoarticular diseases
- 2:45pm Questions & Answers
- 3:00pm Overview:
 - **Philippe Menasché**, Paris Cité University Cell Therapy of Heart Failure: Cells, Exosomes, RNAs?

3:20pm Shot talks:

- **Grégory Lavieu**, Inserm Extracellular Vesicle-based vector for targeted therapeutics delivery
- **Jérémie Laurent**, Astraveus Lakhesys, the microfluidics powered benchtop factory for cell & gene therapies to develop better products and solve scalability
- Jérome Bonnet, Inserm Programming bacteria as smart therapeutics

3:50pm Questions & Answers

4:10pm Keynote:

• Alain Fischer, Professor at the College of France, President of The French Academy of Sciences

Conclusive Keynote: 4:30pm

Marc Bonneville, Scientific and medical director of Merieux Institut, President of ARIIS

B2B Meetings: 4:30pm - 6:00pm