

MORNING PROGRAM

- ▶ 8.00 - 9.00 **Welcome**
- ▶ 9.00 - 9.35 **Official Allocutions**
 - ▶ **Claude Bertrand**, President, ARIIS
 - ▶ **Yves Lévy**, President, AVIESAN
 - ▶ **Thierry Mandon**, *secrétaire d’État chargé de l’Enseignement supérieur et de la Recherche*
 - ▶ **Marisol Touraine**, *ministre des Affaires sociales, de la Santé et des Droits des femmes*
- ▶ 9.35 - 9.45 **Scientific Context and French Organization of Genomic Medicine**
Thierry Frebourg, Co-Director, Institute of Genetics, Genomics and Bioinformatics, AVIESAN
- ▶ 9.45 - 10.45 **Deciphering the Mechanisms of Common Diseases from Rare Diseases**
 - ▶ 9.45 - 9.55 Ciliopathies: from a vestigial organelle to rare diseases and innovation - **Hélène Dollfus**, Inserm, National Reference Centre for Rare Eye Diseases, University Hospital, Strasbourg
 - ▶ 9.55 - 10.05 From extreme phenotypes to new insights in pathophysiology and treatment of autoimmune diseases - **Pierre Ronco**, Inserm, National Reference Centre for Nephrotic Syndrome, Tenon University Hospital, Paris
 - ▶ 10.05 - 10.15 **Q&A**
 - ▶ 10.15 - 10.25 Alzheimer’s disease: from early-onset cases to preventive strategies - **Gaël Nicolas**, Inserm, National Reference Centre for young Alzheimer’s disease patients, University Hospital, Rouen
 - ▶ 10.25 - 10.35 Susceptibility/resistance to common microbes: a genetic theory of infectious diseases - **Laurent Abel**, Inserm, Imagine Institute, Paris
 - ▶ 10.35 - 10.45 **Q&A**
- ▶ 10.45 - 11.05 **Break**

▶ 11.05 - 12.15

Multi-Technological Therapeutic Approaches of Rare Diseases

- ▶ 11.05 - 11.15 Gene therapy of primary immunodeficiencies, safety, efficacy, sustainability - **Alain Fischer**, Inserm, Imagine Institute, Paris
- ▶ 11.15 - 11.25 Towards tailored gene therapy in central nervous system diseases - **Patrick Aubourg**, Inserm, Kremlin-Bicêtre University Hospital, Paris
- ▶ 11.25 - 11.35 Translational approaches of neuromuscular disease using RNA modification - **Thomas Voit**, Inserm, Institute of Myology, Pitié-Salpêtrière University Hospital, Paris
- ▶ 11.35 - 11.45 **Q&A**
- ▶ 11.45 - 11.55 Gene-independent therapies in genetic retinal degenerations - **José-Alain Sahel**, Inserm, Vision Institute, Paris
- ▶ 11.55 - 12.05 Drug discovery using assays based on human pluripotent stem cells - **Marc Peschanski**, Inserm, I-Stem, Évry
- ▶ 12.05 - 12.15 **Q&A**

▶ 12.15 - 12.25

The French Foundation for Rare Diseases

- ▶ **Nicolas Lévy**, Inserm, University Hospital, Marseille

▶ 12.25 - 12.30

Closing of the Morning Session and Perspectives Thierry Frebourg and Claude Bertrand

▶ 12.30 - 2.00

Lunch