THE GENE THERAPY REVOLUTION UNDERWAY: THE LATEST ADVANCES

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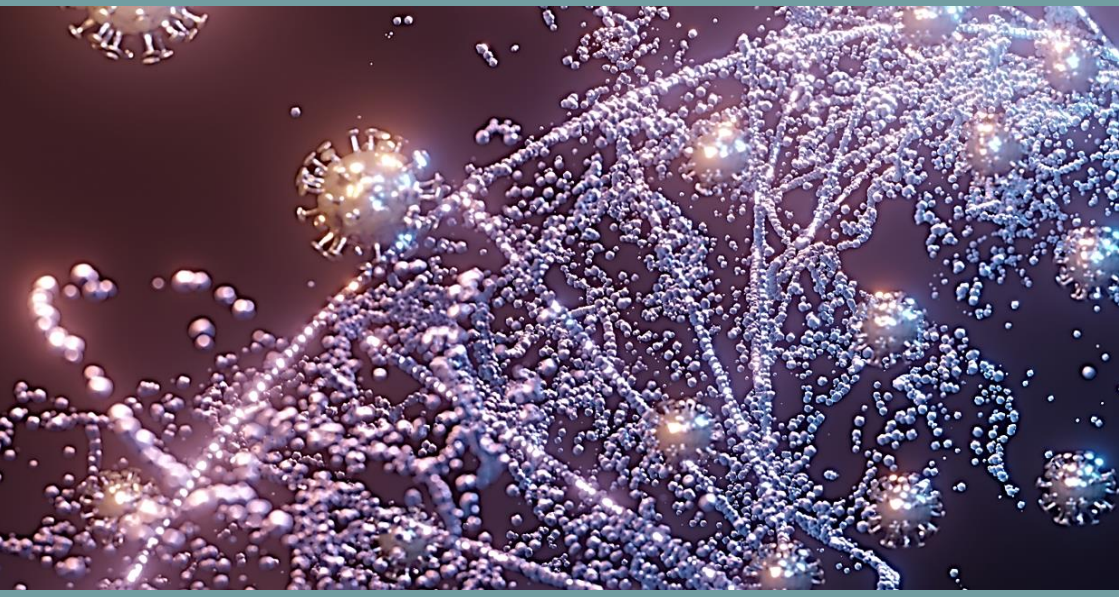

PROGRAM

Institut *Imagine*
Necker Campus
24 Boulevard du Montparnasse
75 015 Paris, France

Event co-organized by

dim Thérapie Génique

institut imagine
GUÉRIR LES MALADIES GÉNÉTIQUES

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THE GENE THERAPY REVOLUTION UNDERWAY: THE LATEST ADVANCES

ABOUT US

Over the last twenty-five years, academic research has been one of the major driving forces behind the spectacular development of gene therapy for rare or acquired diseases. During the last 5 years, Paris Region scientists federated in a network to accelerate the development of gene therapy to reinforce, in the light of personalized medicine, it grades among the most promising and innovative therapeutic strategies and provide treatment for serious or chronic pathologies with unmet medical needs.

The Gene Therapy Area of Major Interest network (DIM Thérapie Génique) organizes **The Gene Therapy Revolution Underway: the latest advances**, a scientific symposium dedicated to recent knowledge and progress in the field to ensure faster and more efficient development of therapies. With 200 intended key actors, this one-day event is the best opportunity to discover the latest gene therapy trends and to see emerge high-potential collaborations.

WHAT IS THE DIM THÉRAPIE GÉNIQUE?

The Paris Region is a pioneer in the field of gene therapy with the first gene therapy protocol to treat children with severe immunodeficiency (X-SCID), developed in 1999 by Pr. Alain Fischer, Marina Cavazzana and Salima Hacein-Bey, and launched at the Necker–Enfants malades Hospital.
The Area of Major Interest (DIM) is the result of a structuring policy of the Paris Region to foster innovation by leveraging the excellence of Paris Region teams. The DIM Thérapie Génique was labelled in 2017 among 13 other DIM for a time-period of 5 years (2017-2021).

Coordinated by Pr. Marina Cavazzana, the DIM Thérapie Génique is a network of public and private stakeholders with the aim to accelerate the development of gene therapy to reinforce, in the light of personalized medicine, it grades among the most promising and innovative therapeutic strategies. The financed research projects in the field of gene therapy aim to bring multidisciplinary expertise with the prospect of clinical trials and to develop promising therapeutic approach becoming innovative solutions available for patients.

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DIM TRACK RECORD

12 M€ of funding from Paris Region for 5 years dedicated to innovative project in gene therapy

15 filed patents in gene therapy

> 10 industrial collaborations
Phasics, Sensorion, Smart Immune, Treefrog, White Lab Genomics, Yposkesi…

1 spin off created
Gamut Therapeutics
acquired in 2021 by SparingVision

56 supported projects
& 33 recruitments

42 publications
driven by researchers from DIM network in high impact journals

> 25 M€ raised
from industrial collaborations and international grants

1 startup creation
on metabolic diseases currently under discussion

For more information: www.dim-tg.org

INSTITUT *IMAGINE*

Established in 2007, the Institut *Imagine* was accredited with “University Hospital Institute” (IHU) status in 2011 under France’s major Investments for the Future program. Its missions – patient-focused research, innovative care, education, training, and technology transfer – have the common goal of establishing better treatment and innovation in care pathway for patients with genetic diseases. Institut *Imagine* is composed of 24 research labs, 4 adjunct laboratories, 31 rare diseases reference centers and 13 core facilities. A critical mass of experts – 1000 scientists, clinicians and healthcare professionals – all established in one place in the Imagine building and the Necker-Enfants malades hospital campus, allow Institut *Imagine* to benefit from key expertise in developmental defects, immunology, infectiology, hematology, nephrology, metabolic diseases, dermatology, gastroenterology, cardiology.

The vision of Institut *Imagine* is to integrate research into clinical practice in order to better understand genetic diseases with unmet medical needs, and to propose and validate diagnostic and therapeutic approaches in partnership with all institutions and founders involved. The study of genetic diseases is fed by knowledge of fundamental biological mechanisms, and in reverse may uncover novel biological concepts. Institut *Imagine* has also developed a strong partnership dynamic with the pharmaceutical industry or innovative health companies and start-ups.

At the forefront of experimental and clinical expertise in gene therapy, Institut *Imagine* manages the Gene Therapy Area of Major Interest (DIM Thérapie Génique).

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PROGRAM

9:00 **INTRODUCTION**

Pr. Stanislas LYONNET, Director of Institut *Imagine*

Pr. Marina CAVAZZANA, Coordinator of the DIM Thérapie Génique

Paris Region representative

MORNING SESSION

CHAIRMEN

Annarita MICCIO, Lab Director, Laboratory of Chromatin and gene regulation during development, INSERM UMR 1163, Institut *Imagine*, Paris

Matthias TITEUX, Researcher, Laboratory of Genetic skin diseases: from disease mechanism to therapy, INSERM UMR 1163, Institut *Imagine*, Paris

9:30 **OVERVIEW OF CHALLENGES AND LATEST PROGRESS IN GENE THERAPY**

Federico MINGOZZI, PhD, Chief Science & Technology Officer, Spark Therapeutics

Title: Progress and challenges in the development of in vivo gene therapies with AAV vectors

10:00 **METABOLIC AND MITOCHONDRIAL DISEASES**

Manuel SCHIFF, MD, PhD, clinician scientist and pediatrician. Reference center for inborn errors of metabolism and Laboratory of Genetics of Mitochondrial diseases, INSERM UMR 1163, Necker-Enfants malades University Hospital and Institut *Imagine*, Paris

Clément PONTOIZEAU, MD, PhD, associate professor of biochemistry, Metabolic biochemistry Unit and Laboratory of Genetics of Mitochondrial diseases, INSERM UMR 1163, Necker-Enfants malades University Hospital and Institut *Imagine*, Paris

Title: AAV-liver gene therapy for Maple Syrup Urine Disease (MSUD)

Agnès RÖTIG, Research Director, Laboratory of Genetics of Mitochondrial diseases, INSERM UMR 1163, Institut *Imagine*, Paris

Title: First steps in gene therapy for Friedreich ataxia

Benedetta RUZZENENTE, Senior research associate, Laboratory of Genetics of Mitochondrial diseases, INSERM UMR 1163, Institut *Imagine*, Paris

Title: Preclinical gene therapy for a mitochondrial disease associated with LRPPRC mutations

11:00 **COFFEE BREAK**

11:30 **BETA-HAEMOGLOBINOPATHIES**

Nicolas HEBERT, Researcher, Laboratory of Transfusion and Pathologies of the Red Blood Cell, INSERM U955, UPEC, EFS, Henri Mondor Hospital, Creteil

Title: Development of functional correction assays for beta-hemoglobinopathies

Annarita MICCIO, Lab Director, Laboratory of Chromatin and gene regulation during development, INSERM UMR 1163, Institut *Imagine*, Paris

Title: Genome editing strategies for beta-hemoglobinopathies

12:30 **LUNCH BREAK**

AFTERNOON SESSION

CHAIRMEN

Vincent MOULY, Research Director, Sorbonne Université, INSERM UMRS 974, Institut de Myologie, Centre de Recherche en Myologie, Pitié-Salpétrière, Paris

Olivier GOUREAU, Research Director, Institut de la Vision, Sorbonne Université, INSERM UMRS 968, CNRS, Paris

13:30 **TOLERANCE, IMMUNITY AND INFLAMMATION**

Julien ZUBER, Professor of Clinical Immunology, Paris Cité Université, Paris

Title: Induction of immune tolerance through gene therapy

Emmanuelle SIX, Research Scientist, Laboratory of Human Lymphohematopoiesis, INSERM UMR 1163, Institut *Imagine*, Paris

Title: HSPC inflammation and gene therapy

14:30 **NEUROMUSCULAR DISEASES**

Judith MELKI , Professor Emeritus, INSERM UMR 1195, Paris-Saclay University, Paris

Title: Circulating cell-free SMN1 nucleic acid in response to gene therapy of spinal muscular atrophy

Isabelle DESGUERRE, MD, PhD, Head of the Pediatric Neurology Unit, APHP-Necker hospital, Filnemus Network for Neuromuscular disorders, Cité Paris Université, Institut *Imagine*, Paris

Title: Gene Therapy in neuromuscular disorders: the example of the SMA in the real live

15:30 **COFFEE BREAK**

16:00 **SENSORY DISORDERS**

Deniz DALKARA, Research Director, Institut de la Vision, INSERM UMRS 968, Paris

Title: Innovative therapies for retinal dystrophies

Christine PETIT, Professor, Auditory Therapies Innovation Lab, INSERM UA06, Department of Hearing Institute – Institut Pasteur Center, Paris

Title: 10 years of preclinical gene therapy for deafness: outcomes and perspectives

17:00 **FROM BENCH TO BEDSIDE: HOW TO ENSURE SAFETY AND EFFECTIVENESS OF GENE THERAPY?**

Alice CORSIA, MD, PhD student, pediatrician, assistant head of clinic, Biotherapies and Therapeutic Hemapheresis Department, Necker-Enfants malades University Hospital, AP-HP, Institut *Imagine*, Paris

Title: Hematological malignancies after Gene Therapy for Sickle Cell disease : how to improve safety?

17:30 **CONCLUSION**

INVITED SPEAKER

Federico MINGOZZI

PhD, Chief Science & Technology Officer,
Spark Therapeutics

Dr. Federico Mingozzi began his scientific career at the University of Ferrara, Italy, where he received his bachelor's degree in biology and his Ph.D. in biochemistry and molecular biology. As a postdoc at the Children's Hospital Philadelphia (CHOP) he conducted studies on liver gene transfer with adeno-associated virus (AAV) vectors and immune tolerance. He contributed to several preclinical and first-in-human clinical studies of gene therapy based on the AAV vector platform while serving as the director of translational research the Center for Cellular and Molecular Therapeutics, at CHOP. He then joined the French National Institute of Health and Medical Research (INSERM) as Research Director and Genethon, a French nonprofit R&D organization focused on gene therapy for rare diseases, as Team Leader. There, he developed *in vivo* gene therapies for inherited diseases, including Crigler-Najjar syndrome, Pompe disease and other glycogen storage diseases. He served as faculty at the Pierre and Marie Curie University in Paris, France, and Universitat Autonoma de Barcelona, Spain, and is currently the Chief Science & Technology Officer of Spark Therapeutics.

PARTNERS


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