# THE GENE THERAPY REVOLUTION UNDERWAY: THE LATEST ADVANCES

**NOVEMBER 16th, 2022** 



# REGISTRATION my.weezevent.com/GTRU

Institut *Imagine*Necker Campus
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# THE GENE THERAPY REVOLUTION UNDERWAY: THE LATEST ADVANCES

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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.

# THE PROGRAMM INCLUDES:

- An overview of the challenges and latest progress in gene therapy
- Presentations of research activities and major achievements from the Gene Therapy Area of Major Interest initiative research teams

## WHY PARTICIPATE?

- Meet international key actors in the field of gene therapy
- Discover the latest trends in the field
- Get an overview of the most innovative ongoing research projects led by Paris Region academic groups under the DIM Thérapie Génique initiative

# **TARGET AUDIENCE:**

- Biotech/medtech and pharma companies
- Academic researchers and physicians

This event is organized by the DIM Thérapie Génique, with the support of Paris Region IIe-de-France

# PRELIMINARY PROGRAM



## **MORNING SESSION**

Chairmen: Annarita MICCIO & Matthias TITEUX

9:00 INTRODUCTION

By Stanislas LYONNET, director of Institut Imagine, and Pr. Marina CAVAZZANA, Coordinator of the DIM Gene Therapy, and Paris Region representative

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

Presentation by Federico MINGOZZI, Chief Scientific Officer, Spark Therapeutics

10:00 METABOLIC AND MITOCHONDRIAL DISEASES

Presentations by Manuel SCHIFF, Clément PONTOIZEAU and Agnès RÖTIG

- 11:00 COFFEE BREAK
- 11:30 BETA-HAEMOGLOBINOPATHIES

Presentations by Annarita MICCIO and Nicolas HEBERT

12:30 LUNCH BREAK

#### AFTERNOON SESSION

Chairmen: Vincent MOULY & OlivierGOUREAU

14:00 TOLERANCE, IMMUNITY AND INFLAMMATION

Presentations by Julien ZUBER and Emmanuelle SIX

15:00 NEUROMUSCULAR DISEASES

Presentations by Judith MELKI and Isabelle DESGUERRE

- 15:30 COFFEE BREAK
- 16:00 SENSORY DISORDERS

Presentations by Deniz DALKARA and Christine PETIT

17:00 FROM BENCH TO BEDSIDE: HOW TO ENSURE SAFETY AND EFFECTIVENESS OF

**GENE THERAPY?** 

Presentation by Laure JOSEPH

17:30 CONCLUSION

# SUPPORTED BY



#### **DIM THERAPIE GENIQUE**

This event is organized by the « DIM Thérapie Génique » (Gene Therapy Area of Major Interest) initiative coordinated by Pr Marina Cavazzana, a network of public and private stakeholders labelled by the Paris Region in 2017 for a time-period of 5 years to accelerate the development of gene therapy. The funded 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.

More info: www.dim-tg.org



#### **INSTITUT IMAGINE**

Institut *Imagine* is a research and innovative healthcare institute of a new type. Located on the campus of the Necker-Enfants malades Hospital in Paris, it brings together, around the patients, over 1 000 researchers, physicians and healthcare professionals, with the aim to better understand, diagnose, and treat genetic diseases. At the forefront of experimental and clinical expertise in gene therapy, Imagine Institute manages the DIM "Thérapie Génique" network.

More info: www.Institutimagine.org



### PARIS REGION ILE DE FRANCE

With a leading economic position in Europe with more than 500 000 companies, 17 of the most prestigious universities in the word, 8 competitiveness clusters and 150 000 researchers, the Paris Region Ile-de-France is one of the most competitive regions in terms of research and development, innovation and entrepreneurship.

More info: www.iledefrance.fr