1ST EDITION

SAVE THE DATE

Feb. 7th & 8th 2019
PARIS

GTRD

Gene Therapy
for Rare monogenic
Diseases

Imagine Institute of Genetic Diseases
Necker Campus
24, boulevard de Montparnasse
75015 Paris, France

www.gtrd-event.com

Event co-organized by :

imagine
INSTITUTE FOR GENETIC DISEASES

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GENE THERAPY FOR RARE MONOGENIC DISEASES

Feb. 7th & 8th 2019

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. Due to the success of recent clinical trials of the last generations of gene therapy products to treat rare genetic diseases and to favorable orphan drug legislation in both Europe and the United States, the biotechnology and pharmaceutical industry is now heavily investing in the field, giving hope to become standard clinical practice.

The Gene Therapy Area of Major Interest (DIM Gene Therapy) launches the first edition of **Gene Therapy for Rare Monogenic Diseases**, an international scientific symposium dedicated to recent knowledge and progress in the field to ensure faster and more efficacious development of therapies for diseases with high unmet medical needs. With 200 intended key actors, this two-day event is the unique opportunity to discover the latest gene therapy trends and to see emerge high-potential collaborations.

THE PROGRAM INCLUDES

- Conferences with international high-level speakers from Europe and the US, mainly focused on clinical gene therapy
- Presentations of research activities and major achievements from the Gene Therapy Area of Major Interest initiative research teams in the larger context of the recent progress in the field
- Posters on the latest gene therapy trends and technologies

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 Gene Therapy Area of Major Interest initiative

PRELIMINARY PROGRAM

DAY 1

13:00	**WELCOME**
13:30	**Introduction of the journey** by Stanislas LYONNET, director of the *Imagine* Institute, and Arnold MUNNICH, President of the *Imagine* Institute
14:00	**Conferences on Lysosomal Storage and Neuromuscular Diseases** Presentations by Alessandra BIFFI (Harvard Medical School), Kevin FLANIGAN (Nationwide Children's Hospital) and Fulvio MAVILIO (Audentes Therapeutics)
15:30	**COFFEE BREAK & POSTER SESSION**
16:00	**DIM Gene Therapy presentations** Discover ongoing research projects in the field of mitochondrial disorders, deafness, retinal dystrophies and corneal stem cell deficiencies
17:30-19:00	**COCKTAIL RECEPTION**

DAY 2

9:00	**Conferences on Haemophilia and AAV immunity** Presentations by Olivier DANOS (RegenX Bio), Federico MINGOZZI (Spark Therapeutics) and Amit NATHWANI (University College London)
10:30	**COFFEE BREAK & POSTER SESSION**
11:00	**Conferences on Haematological diseases** Presentations by Juan BUEREN (CIEMAT), Giuliana FERRARI (San Raffaele-TIGET) and Jean-Antoine RIBEIL (Bluebird Bio)
12:30	**LUNCH BREAK**
14:00	**The Prospect of Gene Editing** Keynote address on gene editing for hemoglobinopathies by Matthew PORTEUS (Stanford University)
14:45	**DIM Gene Therapy presentations** Discover ongoing research projects in the field of gene editing
15:30	**COFFEE BREAK & POSTER SESSION**
16:00	**DIM Gene Therapy presentations** Discover ongoing research projects in the field of sickle-cell disease and CAR-Treg
17:00	**Final lecture and conclusion** by Marina CAVAZZANA, Coordinator of the DIM Gene Therapy
17:30	**END OF THE EVENT**

Registration & more info: **www.gtrd-event.com**

DIM GENE THERAPY SPEAKERS

- **Pablo BARTOLUCCI**,
  Transfusion and Pathologies of the Red Blood Cell, EF
- **Deniz DALKARA**,
  Gene therapies and animal models for neurodegenerative diseases, Institut de la Vision
- **Wassim EL NEMER**,
  Integrated biology of red blood cells, Inserm
- **Olivier GOUREAU**,
  Retinal development and repair, Institut de la Vision
- **Annarita MICCIO**,
  Chromatin and gene regulation during development lab, *Imagine* Institute
- **Makoto MIYARA**,
  Center of Immunology and Infectious Diseases (CIMI Paris), La Pitié-Salpêtrière Hospital
- **Christine PETIT** and **Saaid SAFIEDDINE**,
  Genetics and physiology of hearing lab, Institut Pasteur
- **Agnès RÖTIG**,
  Genetics of mitochondrial diseases Lab, *Imagine* Institute
- **Valérie VANNEAUX**,
  Cellular Therapy Unit, Cord Blood Bank, Saint-Louis Hospital

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