

## IMAGINE - Institut des maladies génétiques

Within Necker-Enfants malades Hospital in Paris, *Imagine* Associated Carnot's main strength is to bring together, on a single site, a large number of genetic diseases' specialists with the aim to better understand these disorders to better diagnose and treat them.

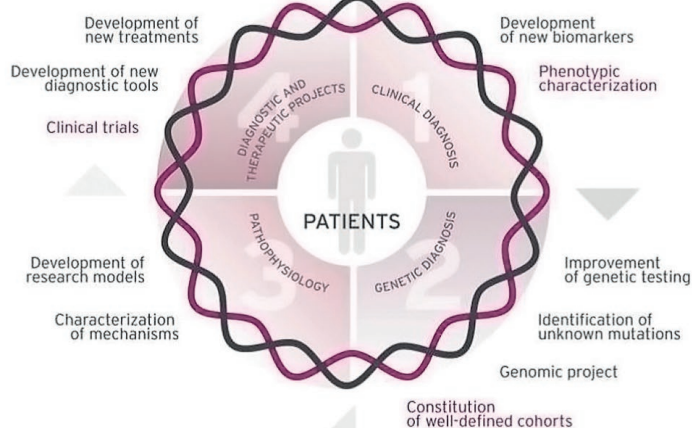
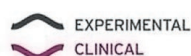
Its patient-centered organization and the close collaboration between clinicians and researchers implement a unique translational model that facilitates research collaborations and the establishment of new therapeutic strategies with the industrial partners.



## An Associated Carnot to better understand, diagnose and treat genetic diseases by associating all the stakeholders

### Addressed market

- Pharmaceutical
- Biotechnology
- Gene and cell therapies
- Diagnostics
- Medical devices

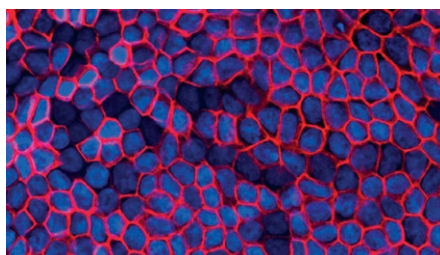


### A translational research institute dedicated to genetic diseases

- ➔ **Clinical expertise:** 510 healthcare professionals, 8 clinical units, 15 Rare Diseases Reference Centers, 2 Clinical Investigation Centers, and 1 data warehouse
- ➔ **Scientific expertise:** 438 researchers, 27 research laboratories, and 3 associated labs
- ➔ **13 core facilities:** data science, transgenesis (CRISPR-Cas9), iPSC cell, biobank, histology, translational genetics, genomic, cytometry, animal facilities, gene transfer, cell imaging, proteomic, and bioinformatics
- ➔ **6 Integrated Care and Research Programs:** Immunology / Hematology / Infectiology / Cardiology, Nephrology, Gastroenterology, Neurosciences / Psychiatry, and Development / Morphogenesis

### Achievements

- **University Hospital Institute** since 2011
- **530 scientific publications in 2016** in several therapeutic areas
- More than **80 clinical studies** involving Tremplin Imagine teams
- **38 active patent families (31/12/2016)**
- **M2/MSc Imagine Bioentrepreneurs** education program with Paris-Descartes University, HEC and Ecole Polytechnique
- Coordination of the 2017-2020 Area of Major Interest « **Gene therapy** » of Paris Region





## Skills and expertise

### • Clinique

Natural history, phenotypic characterization, cohort study

### • Genetic

Genomics and bioinformatics, phenotypic/genotype correlation, identification of new relevant therapeutic targets

### • Preclinical research

New cell and animal models (iPSC, CRISPR-Cas9), cellular and molecular mechanisms characterization, gene and cell therapies, establishment of preclinical and clinical Proof of Concept

### • Clinical research

Clinical project management (including clinical trials and orphan designation), interaction with regulatory agencies, operational and logistical support during the studies, decision support (databases, clinical expertise) enabling the identification and the targeted recruitment of patients

## Partnerships at the heart of our strategy

- Research collaborations, fee for services, start-up creation,
- on preclinical, translational or clinical projects,
- to discover new molecules, repurpose, develop gene or cell therapies,
- with SMEs, biotech and pharmaceutical companies: Alexion, Ascendis, Biomarin, Bluebirdbio, Celgene, Cydan, Genomic Vision, Genzyme, GSK, Inaterys, Novartis, Sanofi, Shire, UCB...

## Objectives

- To better understand physiopathological processes of rare genetic diseases
- To transform know-how into diagnostic and therapeutic solutions to the advantage of the patient as quickly as possible,
- To increase the number of innovative therapeutic studies and the number of patients participating to these studies

Research staff  
(full-time equivalent): **438**  
including PhD students: **82**

Partnership income  
with industry: **5,3 M€**  
Global budget: **51 M€**

## CONTACT

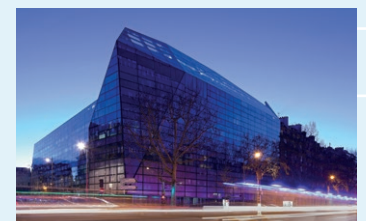
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## Components of Imagine Associated Carnot

- IHU *Imagine* supported by FCS **Imagine**
- UMR1163 (INSERM / Paris Descartes University)



[www.instituts-carnot.eu](http://www.instituts-carnot.eu)