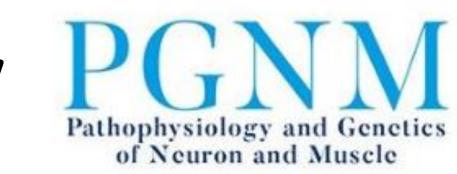


# 'iPS\_PGNM' core facility





Institut NeuroMyoGene - Pathophysiology and Genetics of Neuron and Muscle (INMG-PGNM) UCBL - CNRS UMR5261 - INSERM U1315

Faculté de Médecine et de Pharmacie de Rockefeller, 8 avenue Rockefeller 69008 Lyon – France Risson V., Grosjean I., Beccari L., Gache V., Lomonte P., Puccio H. and Schaeffer L.





The objective of the PGNM laboratory is to elucidate fundamental aspects of the cell biology of muscle and the nervous system, from development to aging, under normal and pathological conditions.

The iPS PGNM is a service under the supervision of the CNRS, INSERM and the University Claude Bernard Lyon1. The facility manages services related to the manipulation of cells used for research in biology and health, in particular induced Pluripotent Stem Cell (iPSCs). It benefits from access to the specific equipment and expertise of the INMG-PGNM to develop new collaborative projects and from the expertise of the Hospices Civils de Lyon (HCL) for karyotypes analysis and identification of genetic anomalies. Its objective is to encourage scientific cooperation and the exchange of technical data concerning the use of iPSCs.

## **SERVICES**

The iPS PGNM facility is open to all internal and external personnel, academic and industrial actors, who wish to carry out scientific projects in the field of iPSCs.

The facility stores, maintains and monitors iPS cell lines.

To carry out its mission, the department offers: technical support and know-how on methods and expertise in cell culture of primary cells and cell lines, traceability and quality control of cells.

It also provides scientific and technical advice to users concerning the differentiation of iPSCs into neurons, cardiomyocytes, spheroids and organoids. It also manages the generation of genetically modified iPS clones using gene editing technology.

#### **EQUIPMENTS**

The facility uses a biosafety level 2 cell culture laboratory equipped with 3 Biological Safety Stations, 2 incubators, 1 microscope, 1 Makrolite.

#### CELL CULTURE TRAINING

The facility also offers a variety of training services to meet the different demands of the scientific community in a very structured way: cell line culture training (2 days), iPSCs culture training (3 days), 3D culture training (3 days). All training courses combine theory and practice.

CONTACTS: team.ips@univ-lyon1.fr, 06 88 74 09 83

**Laurent Schaeffer** Responsible

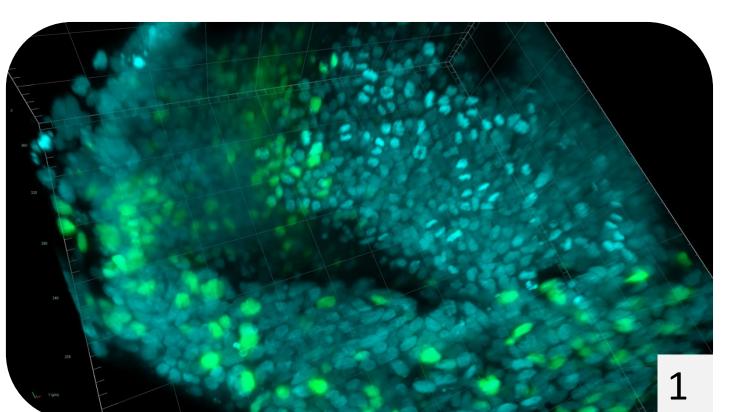
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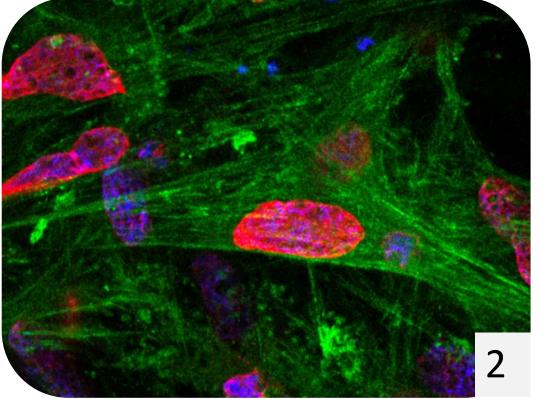
Valérie Risson

Scientific Coordinator valerie.risson@univ-lyon1.fr Isabelle Grosjean,

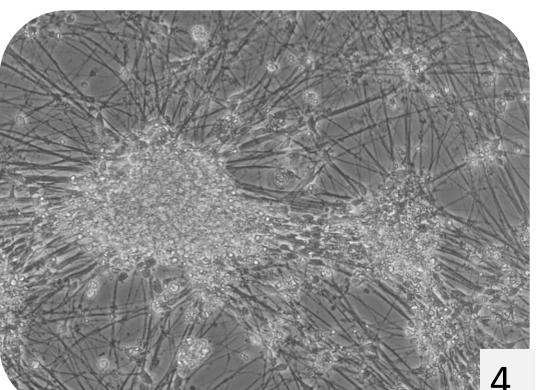
Technical manager

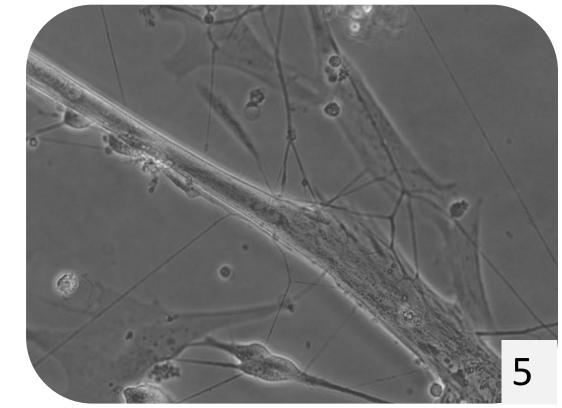
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1) Cerebral organoid mosaic for transgenic cells carrying a GFP reporter construct under the control of a regulatory sequence active in all neural precursors © Léonardo Beccari. 2) Cardiomyocyte derived from iPSC © Estèle Lafont. 3) Microfluidic Pattern for sensory neurons © Simon Roubille. 4) Human iPS cell differentiation into sensory neurons, day19 © Valentine Mosbach. 5) Muscle-Motoneuron co-culture © Julien Carras.

## iPS\_PGNM team USERS

### Beccari Léonardo

I combine the use of human brain organoids with CRISPR- genome editing in IPS cells to understand how the expression of genes involved in cortex development is regulated and how mutations in non-coding sequences controlling their activation lead to neurodevelopmental brain disorders in humans.

### **Gache Vincent**

« Cardiomyopathy is an heterogenous group of cardiac disorders with alteration of heart tonus. There are different types (dilated, hypertrophic, restrictive,...). In different cases, the cardiac disorders can be associated to a muscular dysfonction. To date, many genes have been identified to be implicated. To discover the physiopathological mechanism, we use cardiomyocyte derived from iPSc generate from patient PBMC (Peripheral Blood Mononuclear Cell). We develop the CRISPR strategy to produce iPSC mutated and HiPSC Wild Type ».

## Lomonte Patrick

- « The loss of Survival Motor Neuron (SMN) gene causes Spinal Muscular Atrophy (SMA). We use human iPS cells derived from a SMA patient to generate motor neurons as a cellular model to investigate the role of SMN in the SMA pathology ».
- « Herpes Simplex Virus 1 is a neurotropic virus responsible for neurons decay. We differentiate human iPS cells into sensory neurons and adapt them in microfluidic devices as a model to investigate HSV-1 viral infection »

### Puccio Hélène

Fe-S cluster biogenesis, an essential process producing inorganic cofactors for a variety of proteins involved in key cellular pathways, is performed by a multiprotein machinery (ISC) including the scaffold protein ISCU, where the iron and sulfur is assembled, and the regulatory protein frataxin (FXN). Reduced FXN levels leads to Friedreich's ataxia, a rare neurodegenerative disease where specific sensory neurons, the proprioceptive neurons, are affected. We are using iPS cell to engineer, thanks to CRISPR strategy, mutations in the ISC complex to better understand after differentiation into sensory neurons Fe-S clusters biogenesis.

# Schaeffer Laurent

In the team, we use motoneurons derived from iPSCs generated from patients or controls to investigate neurodegenerative disorders such as Charcot-Marie-Tooth diseases or neuromuscular disorders such as congenital myasthenic syndrome. These motoneurons can be cocultured with immortalized human skeletal muscle stem cells providing a tool to study features of neuromuscular jonction formation.