Research evaluation

FINAL RESUME ON THE RESEARCH UNIT

Thérapie génique translationnelle des maladies génétiques

UNDER THE SUPERVISION OF THE FOLLOWING INSTITUTIONS AND RESEARCH BODIES:

Université de Nantes
Institut national de la santé et de la recherche médicale - INSERM

EVALUATION CAMPAIGN 2020-2021
GROUP B

Report published on October, 20 2021

High Council for evaluation of research and higher education

In the name of Hcéres[1]:	In the name of the experts committee[2]:
Mr Thierry Coulhon, President	Ms Hélène Puccio, Chairwoman of the committee

Under the decree No.2014-1365 dated 14 November 2014,

[1] The president of Hcéres "countersigns the evaluation reports set up by the experts committees and signed by their chairman." (Article 8, paragraph 5);

[2] The evaluation reports "are signed by the chairman of the experts committee". (Article 11, paragraph 2).

Tables in this document were filled with certified data submitted by the supervising body on behalf of the unit.

UNIT PRESENTATION

Unit name:
Thérapie génique translationnelle des maladies génétiques
Unit acronym:
Thérapie génique translationnelle des maladies génétiques
Current label and N°:
1089
ID RNSR:
200416339X
Application type:
Renewal
Head of the unit (2020-2021):
Ms Oumeya Adjali
Project leader (2021-2025):
Ms Oumeya Adjali

EXPERTS COMMITTEE MEMBERS

Chair:	Ms Hélène Puccio, Institut de Génétique et de Biologie Moléculaire et Cellulaire UMR 7104/U1258, Illkirch and Institut Neuromyogène, UMR5310/U1217, Lyon
Experts:	Ms Maité Carre-Pierrat, Institut Neuromyogène, UMR5310/U1217 Lyon Mr Luis Garcia, UMR 1179 INSERM-Université de Versailles Saint Quentin, Montigny-Le-Bretonneux

HCÉRES REPRESENTATIVE

M. Hinrich Gronemeyer

REPRESENTATIVES OF SUPERVISING INSTITUTIONS AND BODIES

Mr Frank Boury, Doctoral School
Mr Bertrand Cariou, Nantes University
Mr Olivier Grasset, Nantes University
Ms Marie-Josèphe Leroy-Zamia, INSERM
Ms Rozenn Tanguy, Nantes University

INTRODUCTION

HISTORY AND GEOGRAPHICAL LOCATION OF THE UNIT

The unit UMR Inserm 1089 "Translational Gene Therapy for Genetic Diseases" is located in "Nantes Biotech/ Institut de Recherche en Santé (IRS2)", a new research center closely linked to the future Nantes hospital (CHU). The unit was created in 2011 to develop ambitious research programs dedicated to gene therapy approaches for neuromuscular and retinal genetic diseases, with a strong translational aspect.

RESEARCH ECOSYSTEM

The laboratory is a unique setting allowing to cover the full spectrum of translational development for gene therapy approaches from vector development, proof-of-concept in animal models to Phase I/II clinical trials including toxicology studies.

The unit strategic position within the Nantes "Pôle Santé" with the close proximity with the future CHU and with the presence of biotechs will further reinforce the translational and clinical activities of the unit.

As an integrated part of the unit, three highly skilled technological facilities have been developed, two of them over the past contract: 1) a vector production core dedicated to AAV manufacturing, 2) a preclinical analytics core with expertise in preclinical evaluation of gene therapy approaches, and 3) a gene therapy immunology core dedicated in monitoring host immune responses. These three facilities are closely linked to the research expertise of the unit and accelerates preclinical gene therapy development.

Two additional key independent facilities are in close interaction with the unit: a GMP-compliant platform for AAV vector production and a dedicated animal facility (Boisbonne gene and cell therapy center) to evaluate gene therapy products in large-size animal models.

The laboratory is involved in different local networks (CAPACITIES, an affiliated private company of the Nantes University for business development; Atlanpole biotherapy competitivity cluster, Bioregate network for regenerative medicine of the Loire region). Locally, the unit is actively involved in the PIA I-SITE initiative of excellence (NExT1 and NExT2 programs). In addition, the unit benefited from several PIA labels (IHU, 'Demonstrateur Pré-Industriel'), and two recently obtained programs PIA SFRI for the graduate school "TRITON" and PIA 'intégrateur industriel' for the Vector production facility.

HCÉRES NOMENCLATURE AND THEMATICS OF THE UNIT

SVE Sciences du vivant et environnement

MANAGEMENT TEAM

The director for the current and next contrat is Ms Oumeya Adjali. The DU is supported by the board of direction comprising the group leaders, the administrative manager and the quality manager. In addition, the unit has a CPV (Centre de Production de Vecteurs) strategic committee and a scientific committee.

UNIT WORKFORCE

Translational Gene Therapy For Genetic Diseases

Active staff	**Number 06/01/2020**	**Number 01/01/2022**
Full professors and similar positions	2	2
Assistant professors and similar positions	2	2
Full time research directors (Directeurs de recherche) and similar positions	1	1
Full time research associates (Chargés de recherche) and similar positions	6	8

Other scientists ("Conservateurs, cadres scientifiques des EPIC, fondations, industries, etc.")	0	
High school teachers	0	
Supporting personnel (ITAs, BIATSSs and others, notably of EPICs)	34	33
Permanent staff	**45**	**46**
Non-permanent professors and associate professors, including emeritus		
Non-permanent full time scientists, including emeritus, post-docs (except PhD students)	3	
PhD Students	5	
Non-permanent supporting personnel	3	
Non-permanent staff	**11**	
Total	**56**	**46**

GLOBAL ASSESSMENT OF THE UNIT

Over the past five years, the unit has achieved a good balance between basic and translational research programs. At the fundamental level, it has launched several fundamental projects aiming at understanding the physiopathology of muscular and retinal diseases, improving AAV vector production methods, characterizing the immune response to enable successful clinical translation of gene therapy developments. It has been successful in recruiting an ATIP-Avenir group which should further strengthen the fundamental research programs.

The unit overall has a very good scientific quality and production. The relatively low publication record in excellent journals, which is a direct consequence of the highly translational aspect of the research of the unit in the rare disease field, is however compensated by the remarkable transfer activity : eight filing patents, four of which have been licensed in the last review period, a very strong interaction with industrial partners with more than 46 contracts executed during the period, the creation of a start-up company, HORAMA, which is dedicated to the clinical translation of ocular gene therapy, two clinical trials (Phase I/II) conducted in the field of genetic retinal diseases and the participation of the GTI/immunology core in five additional clinical trials.

Overall, the unit has excellent collaborative projects with academic partners and very good to excellent funding from charities (GENETHON, AFM, FRM as coordinator), national (3 ANR, 2 IBISA and 1 UNADEV-AVIESAN, all as coordinator) and international (1 MDA (USA) as coordinator, 1 European H2020 as partner) grants, which enables a sustainable financial strategy.

The unit has an internationally recognized leadership in the gene therapy field in particular in rAAV vectorology improvements and preclinical research developing adequate animal models, with a unique expertise in large animal models allowing translation of the preclinical work into clinical phases.

The five-year project is a continuation of the previous research activities. It is based on unique know-how and a set of platforms whose level of excellence is recognized in the field. Beyond its involvement in supporting clinical trials, the unit also proposes to develop a pertinent program on chemical peptide coupling to improve targeting and reduce the immunogenicity of AAV vectors. It is risky but can also constitute an opportunity for a bio-technological breakthrough and future innovations. Aiming at more ambitious research programs would increase the chance in publishing in high-profile journals with broader audience.

The position of the unit within the Nantes "Pôle Santé" is a major asset for the unit to reinforce the translational and clinical activities. The strong support from the "Centre Hospitalier Universitaire de Nantes" in association to Inserm is important. The unit is actively involved in strategic local networks and in several I-SITE initiatives.

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