

FINAL RESUME ON THE RESEARCH UNIT:  
Integrated Genetic Approaches in Therapeutic  
Discovery for Rare Diseases (INTEGRARE)

UNDER THE SUPERVISION OF THE  
FOLLOWING INSTITUTIONS AND  
RESEARCH BODIES:

Université d'Evry-Val-d'Essonne - UEVE  
Institut national de la santé et de la recherche  
médicale – Inserm

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**EVALUATION CAMPAIGN 2018-2019**  
GROUP E

Report published on February, 27 2019



In the name of Hcéres<sup>1</sup>:

Michel Cosnard, President

In the name of the experts committee<sup>2</sup>:

Didier Trono, Chairman of the committee

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

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

<sup>2</sup> The evaluation reports "are signed by the chairman of the experts committee". (Article 11, paragraph 2).

Tables in this document were filled with data provided by laboratories and supervising bodies in the unit's application and in the Excel files "Données du contrat en cours" and "Données du prochain contrat".

## UNIT PRESENTATION

<b>Unit name:</b>	Integrated Genetic Approaches in Therapeutic Discovery for Rare Diseases
<b>Unit acronym:</b>	INTEGRARE
<b>Requested label:</b>	UMR
<b>Application type:</b>	Renewal
<b>Current number:</b>	UMR_S951
<b>Head of the unit (2018-2019):</b>	Ms Anne GALY
<b>Project leader (2020-2024):</b>	Ms Anne GALY
<b>Number of teams:</b>	3

## EXPERTS COMMITTEE MEMBERS

<b>Chair:</b>	Mr Didier TRONO, École Polytechnique Fédérale de Lausanne, Switzerland
<b>Experts:</b>	Ms Julie KERR-CONTE, Université de Lille (representative of CNU) Mr Steve LANCEL, Université de Lille Mr Didier LETOURNEUR, Sorbonne Paris Cité, (representative of Inserm CSS) Mr Nicolas SETTERBLAD, Sorbonne Paris Cité (supporting personnel)

## HCÉRES REPRESENTATIVE

Ms Sophie EZINE

## REPRESENTATIVES OF SUPERVISING INSTITUTIONS AND BODIES

Ms Camille CHAUDONNERET and Ms Corinne SEBASTIANI, Inserm

Mr Patrick CURMI and Ms Florence GONNET, Université d'Evry Val d'Essonne

Mr Frédéric REVAH, Génethon

## INTRODUCTION

### HISTORY AND GEOGRAPHICAL LOCATION OF THE UNIT

The mixed research unit UMR\_S951 "Molecular Immunology and Innovative Therapies" (IMBI) was established as the first Inserm (Institut National de la Santé et de la Recherche Médicale) mixed research unit within Genethon in 2009. Genethon, located in Evry, Southeast of Paris, is a private non-for-profit laboratory created 30 years ago by the French Muscular Dystrophy Association/Telethon (AFM/Telethon).

This research unit, led by Anne Galy, was renewed in 2014 for a 5 year period under the name INTEGRARE (Integrated Genetic Approaches for the Treatment of Rare Diseases).

### MANAGEMENT TEAM

The UMR\_S951 is directed by Anne Galy.

### HCÉRES NOMENCLATURE

SVE5\_3.

### SCIENTIFIC DOMAIN

The unit is covering many specific aspects of gene and cell therapy research programs of Genethon. It is focused on the development of various well-tolerized gene therapy approaches such as liver gene transfer or ex vivo hematopoietic stem cell-based gene therapies for rare diseases; and novel approaches to gene-based therapies for neuromuscular diseases and genome editing, muscular dystrophies and congenital myopathies.

### UNIT WORKFORCE

	Unit workforce	
	Integrated genetic approaches in therapeutic discovery for rare diseases	
Active staff	Number 30/06/2018	Number 01/01/2020
Full professors and similar positions	2	1
Assistant professors and similar positions	2	2
Full time research directors (Directeurs de recherche) and similar positions	3	3
Full time research associates (Chargés de recherche) and similar positions	1	2
Other scientists ("Conservateurs, cadres scientifiques des EPIC, fondations, industries, etc.")	6	6
High school teachers	0	0
Supporting personnel (ITAs, BIATSSs and others, notably of EPICs)	18	18
<b>Permanent staff</b>	<b>32</b>	<b>32</b>

Non-permanent professors and associate professors, including emeritus	0	
Non-permanent full time scientists, including emeritus, post-docs	16	
<i>PhD Students</i>	14	
Non-permanent supporting personnel	6	
<b>Non-permanent staff</b>	<b>36</b>	
<b>Total</b>	<b>68</b>	

## GLOBAL ASSESSMENT OF THE UNIT

This very good unit nicely capitalizes on its unique position at the interface between academia and the implementation-facilitating Genethon. The unit has high international visibility within the community of scientists and medical doctors engaged in developing gene-based approaches for the treatment of hematological and immunological disorders. Accordingly, it is involved in several collaborative clinical trials at the forefront of this pioneering field, albeit in the majority of cases based on fundamental research performed elsewhere. There is a dichotomy between success in this area and more laborious efforts in the field of neuromuscular disorders, although this is partly explained by the formidable therapeutic opportunities offered by the genetic modification of hematopoietic stem cells, without a real counterpart in the muscle field. Scientific productivity is very good and would be strengthened by publications in higher impact journals with the unit's researchers in leading positions. As well, the local landscape could be more formally exploited as training ground for a new generation of physician-scientists engaged in cell- and gene-based therapeutics. The level of comfort provided by Genethon-based funding represents a major financial security for the unit. The long-term leadership of the unit should be examined taking into full consideration the links between its success and the unique profile and talent of its current head.

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