

FINAL RESUME ON THE RESEARCH UNIT:  
Institute of stem cell for the treatment and study  
of monogenic diseases (I-STEM)

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, 01 2019



In the name of Hcéres<sup>1</sup>:

Michel Cosnard, President

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

Bruno Peault, 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>	Institute of stem cell for the treatment and study of monogenic diseases
<b>Unit acronym:</b>	I-STEM
<b>Requested label:</b>	UMR
<b>Application type:</b>	Renewal
<b>Current number:</b>	UMR861
<b>Head of the unit (2018-2019):</b>	Ms Cécile MARTINAT
<b>Project leader (2020-2024):</b>	Ms Cécile MARTINAT
<b>Number of teams:</b>	2

## EXPERTS COMMITTEE MEMBERS

<b>Chair:</b>	Mr Bruno PEULT, University of Edimburg, United Kingdom and University of Los Angeles, United States of America
<b>Experts:</b>	Mr Xavier DECLEVES, Université Paris Descartes (representative of Inserm CSS) Mr Chris DENNING, University of Nottingham, United Kingdom Mrs Isabelle Duroux-RICHARD, Université de Montpellier (supporting personnel) Mr Thierry OSTER, Université de Lorraine (representative of CNU)

## HCÉRES REPRESENTATIVE

Mr Pierre COUBLE

## REPRESENTATIVES OF SUPERVISING INSTITUTIONS AND BODIES

Mr Patrick CURMI, Université d'Évry-Val-d'Essonne  
Ms Florence GONNET, Université d'Évry-Val-d'Essonne  
Ms Corinne SEBASTIANI, Inserm

## INTRODUCTION

### HISTORY AND GEOGRAPHICAL LOCATION OF THE UNIT

I-STEM was launched in 2005 as a joint project, headed by Marc Peschanski, between the French AFM (Association Française contre les Myopathies) and Inserm. At that early stage already, I-STEM was focused on the study of human pluripotent stem cells, and used thereof to treat patients with genetic diseases. The mission and focus of the Institute have remained remarkably unchanged in the past thirteen years. Shortly after the discovery and publication in 2007 of cell reprogramming to stemness, scientists at I-STEM rapidly added iPS cells to their stem cell inventory. Administratively, I-STEM is an Inserm/University of Evry-Val-d'Essonne Joint Research Unit (U861), closely associated, in the same building, with CECS, a Center for Stem Cell Research directly affiliated with AFM.

Since 2015, I-STEM occupies 1600 square meters within the Center for Clinical and Translational Research (CRCT) in Corbeil-Essonnes, about 40 km south of central Paris. In the immediate vicinity are located major French biomedical research organizations: Genopole, AFM-Genethon, as well as numerous biotechnology companies and the University of Evry-Val-d'Essonne.

### MANAGEMENT TEAM

I-STEM/UMR 861 is headed by Ms Cécile Martinat.

### HCÉRES NOMENCLATURE

SVE4\_1; SVE5\_1.

### SCIENTIFIC DOMAIN

Since its creation, I-STEM has combined basic and applied research in order to identify innovative therapies for rare diseases based on the exploration of the potential offered by human pluripotent stem cells. Its global strategy has been elaborated in function of the two main therapeutic applications offered by these cells: cell therapy and drug discovery.

### UNIT WORKFORCE

	Unit workforce	
	INSERM UEVE UMR861	
Active staff	Number 30/06/2018	Number 01/01/2020
Full professors and similar positions	2	2
Assistant professors and similar positions	2	1
Full time research directors (Directeurs de recherche) and similar positions	3	3
Full time research associates (Chargés de recherche) and similar positions	1	0
Other scientists ("Conservateurs, cadres scientifiques des EPIC, fondations, industries, etc.")	0	0
High school teachers	0	0
Supporting personnel (ITAs, BIATSSs and others, notably of EPICs)	4	4

<b>Permanent staff</b>	<b>12</b>	<b>10</b>
Non-permanent professors and associate professors, including emeritus	1	
Non-permanent full time scientists, including emeritus, post-docs	7	
<i>PhD Students</i>	7	
Non-permanent supporting personnel	15	
<b>Non-permanent staff</b>	<b>30</b>	
<b>Total</b>	<b>42</b>	

## GLOBAL ASSESSMENT OF THE UNIT

A common denominator between all research groups at I-STEM is the use of human pluripotent cells as models of rare genetic diseases, assays for therapeutic compounds and sources of transplantable regenerative cells. Targeted pathologies at UMR 861 are neurodegenerative and retinal diseases, skin genetic diseases, and neuromuscular diseases. Equipment and facilities at I-STEM, served by a large staff of highly competent technicians and engineers, are exceptional and can guarantee the highest experimental performance. Indeed, I-STEM had a remarkable record of publications in recent years, with several articles published in very high-ranking journals. The review committee was generally impressed by the quality of the research performed at UMR 861, and by the fact that therapeutic “translation” is a reality at I-STEM, with strong links with the pharmaceutical industry and on-going and imminent trials in patients. There is still space for improvement though, and it was recommended that efforts be made in terms of international PhD students, postdocs and visiting professors recruitment and worldwide promotion of I-STEM production. Regarding basic science, it was suggested that the right balance is to be found between cultured pluripotent cells, which are ideal but somehow simplified models for high-throughput compound screening, and more complex native stem cell models that may reflect more faithfully the physiopathologic reality.

Overall, scientific output, relationship with the non-academic world and research strategies and tactics were always ranked as excellent or outstanding.

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