

REPORT ON THE CIC:
Centre d'Investigation Clinique Intégré en
Biothérapie
(CIC-BT)

UNDER THE SUPERVISION OF THE
FOLLOWING INSTITUTIONS AND
RESEARCH BODIES:
Institut National de la Santé et de la Recherche
Médicale – INSERM
Université Paris Descartes

ÉVALUATION CAMPAIGN 2017-2018
GROUP D



In the name of Hcéres¹:

Michel Cosnard, President

In the name of the expert committee²:

Mirjam Van Der Burg, 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 expert committees and signed by their chairman." (Article 8, paragraph 5);

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

This report is the sole result of the CIC's evaluation by the expert committee, the composition of which is specified below. The assessments contained herein are the expression of an independent and collegial reviewing by the committee.

CIC PRESENTATION

name: Centre d'Investigation Clinique Intégré en Biothérapie

number:

Requested label:

Application type: Renewal

Head of the CIC (2017-2018): Ms Marina CAVAZZANA

Project leader (2019-2023): Ms Marina CAVAZZANA

Number of modules or themes:

COMMITTEE MEMBERS

Chair: Ms Mirjam VAN DER BURG, LUMC – Dept. of Pediatrics, Laboratory for Immunology, Leiden, The Netherlands

Experts: Mr Seiamak BAHRAM, CHU Strasbourg
Mr Pierre COULIE, Université catholique de Louvain, Brussels, Belgium

HCERES scientific officer:
Mr Philippe VANHEMS

Representatives of supervising institutions and bodies:
Ms Samia DEGHMOUN, Inserm
Ms Hélène ESPEROU, Inserm

INTRODUCTION

HISTORY AND GEOGRAPHICAL LOCATION OF THE CIC

The Biotherapy Clinical Investigation Centre (CIC-BT) is an integral part of the Western Paris University Hospital Group (GHU Ouest) composed of Necker Children's Hospital, Cochin Hospital, and Georges-Pompidou European Hospital. It was founded in 2005 with support from the French Directorate General for Healthcare Provision (DGOS), the French Institute National of Healthcare and Medical Research (INSERM), and the French Muscular Dystrophy Association (AFM).

The CIC-BT is located at several sites on the Necker Children's Hospital campus:

- the **Translational research group** is located in the *Imagine* Institute. It is part of the Human Lymphohaematopoiesis Laboratory (located on the fifth floor of the *Imagine* Institute, INSERM Unit U1163). It helps to evaluate ongoing trials and implement preclinical trials, and collaborates closely with the Biotherapy Department's Cell and Gene Therapy Laboratory (LTCG);
- the **Cell and Gene Therapy Laboratory (LTCG)** is also located on the Necker campus in the basement of the Hamburger building on the Necker Children's Hospital campus. Its role is translate basic work into clinical trials;
- the **Immunodeficiency Reference Centre (CEDI)** is an integral part of the CIC-BT; its role is to perform immunological monitoring of patients in ongoing trials and in the Therapeutic Haemapheresis Day Hospital. CEDI is located on the second floor of the Lavoisier building on the Necker Children's Hospital campus;
- the **Biotherapy Department's Therapeutic Haemapheresis Day Hospital** is located on the first floor of the Hamburger building on the Necker Children's Hospital campus;
- the **Mother & Infant CIC and the Clinical Research Unit** is coordinated by Mr Jean-Marc Tréluyer and located on the Necker Children's Hospital campus (not evaluated).

MANAGEMENT MODULE

The director of the CIC-BT is Ms M. Cavazzana; the vice director is Ms I. André-Schmutz.

HCERES NOMENCLATURE

SVE6 Santé Publique, Épidémiologie, Recherche Clinique.

SCIENTIFIC DOMAIN

The CIC-BT's main mission is to develop clinical research studies in the fields of cell and gene therapy for the treatment of acquired and hereditary diseases in patients of all ages, in connection with the research/healthcare themes and priorities on the Necker Children's Hospital campus. The CIC-BT develops new trials in three areas: (i) genetic diseases; (ii) vascular diseases, and (iii) immunohematology.

CIC WORKFORCE

CIC workforce	Number 30/06/2017	Number 01/01/2019
Permanent staff		
Full professors, assistant professors and similar positions	6	6
Clinicians (PH)	5	2

Full time research directors (Directeurs de recherche) or Research associates (chargés de recherché) EPST , EPIC and similar positions	2	1
Full professors, associate professors and clinicians (PH) affiliated to a CNRS, INSERM or EA research CIC		0
Other permanent staff involved in research: health professionals, supporting personnel etc.	4	7
TOTAL permanent staff	17	16
Non-permanent staff		
Non-permanent professors and associate professors, including emeritus,)	0	
Non-permanent full time scientists, including emeritus, post-docs	0	
Non-permanent supporting personnel	22	
PhD Students	0	
TOTAL non-permanent staff	22	
TOTAL CIC		
	39	

GLOBAL ASSESSMENT OF THE CIC

The scientific work of the CIC-BT is outstanding. The quantity and quality of the papers are very high and the papers are published in top ranking journals. The outstanding nature of their work is also illustrated by the high number of academic prizes awarded to the coordinator and several of her team members. It is a challenge to maintain this quality and fruitful strategy. The interactions with outside academia are also outstanding. There are many links with companies, which is important for current and future funding. In addition, the CIC-BT has an open attitude to patient organisations. The grant manager, who has been appointed since the last evaluation visit (2012-2013) is of great support for managing the large number of projects and grants, but also for new grant applications.

Ms Marina Cavazzana, the coordinator, has built an outstanding team of motivated and well respected personnel with a high female labour participation and a very good atmosphere. The CIC-BT is well positioned and integrated in a fairly complex network of organisational structure.

The scientific strategy is outstanding: primary immunodeficiencies are used to explore new biological treatment possibilities that can be exploited in other genetic diseases or in gene therapies for other more frequent conditions, which impact on public health. CIC-BT is a world-leading center with this strategy. Since the last evaluation visit, the team clearly opened their scope to wider range of disease beyond the very rare PID including the ex vivo accelerated T lymphocyte differentiation and proliferation prior to their injection in patients transplanted with hematopoietic stem cells, and a double gene therapy including CCR5 invalidation and CD46 transfer for HIV patients.

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