Research evaluation

EVALUATION REPORT OF THE UNIT

END-ICAP - Handicap Neuromusculaire: Biothérapies et Innovations Technologiques

UNDER THE SUPERVISION OF THE FOLLOWING ESTABLISHMENTS AND ORGANISMS:

Université de Versailles Saint-Quentin-en-Yvelines - UVSQ

Institut national de la santé et de la recherche médicale - Inserm

EVALUATION CAMPAIGN 2024-2025
GROUP E

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High Council for evaluation of research and highter education

In the name of the expert committee:

Ms Odile Boespflug-Tanguy, chairwoman of the committee

For the Hcéres:

Stéphane Le Bouler, acting president

In accordance with articles R. 114-15 and R. 114-10 of the Research Code, the evaluation reports drawn up by the expert committees are signed by the chairmen of these committees and countersigned by the president of Hcéres.

To make the document easier to read, the names used in this report to designate functions, professions or responsibilities (expert, researcher, teacher-researcher, professor, lecturer, engineer, technician, director, doctoral student, etc.) are used in a generic sense and have a neutral value.

This report is the result of the unit's evaluation by the expert committee, the composition of which is specified below. The appreciations it contains are the expression of the independent and collegial deliberation of this committee. The numbers in this report are the certified exact data extracted from the deposited files by the supervising body on behalf of the unit.

MEMBERS OF THE EXPERT COMMITTEE

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Mr Loic Josseron, Dean of UFR de Médecine
Ms Marie-Josèphe Leroy-Zamia, Inserm
Ms Laurence Parmantier, Inserm

CHARACTERISATION OF THE UNIT

- Name: Handicap Neuromusculaire: Biothérapies et Innovations Technologiques
- Acronym: END-ICAP
- Label and number: UMR1179
- Composition of the executive team: Mr Luis Garcia (director), Mr Marcel Bonay (deputy directeur) & Ms Valérie Robin (lab. manager)

SCIENTIFIC PANELS OF THE UNIT

SVE Sciences du vivant et environnement
SVE5 Neurosciences et troubles du système nerveux

THEMES OF THE UNIT

The unit Handicap Neuromusculaire: Biothérapies et Innovations Technologiques (ENDICAP UMR1179) dedicates its research to developing innovative solutions for neuromuscular and neurological disorders. The unit is composed of three different teams, which can be assigned to three main themes:

1. Gene Therapy for neuromuscular and neurological disorders: This theme is led by the efforts of the biotherapies for neuromuscular diseases and neurogenic bladder dysfunctions groups, which has contributed to significant advancements in gene therapy. The work on antisense oligonucleotides (tricyclo-DNA) has contributed to a "first in Human" clinical trial AVANCE 1 (Phase 1/2a), which targets the treatment of Duchenne Muscular Dystrophy (DMD). AVANCE1 is the culmination of years of collaboration between SQY Therapeutics, a company created by two parents' associations (Duchenne Parent Project France & Association Monégasque contre les Myopathies) at the UFR santé Simone Veil (UVSQ), the UMR1179 and the hospital services of the Raymond Poincaré University Hospital (APHP-Garches). The second theme on neurogenic uro-genital disorders focuses on gene therapy solutions using a herpes virus vector (HSV1) for bladder dysfunctions resulting from spinal cord injuries or other neurological conditions. A notable project is ELPIS, which has led to the creation of the startup EG427, which has launched a Phase1/2 clinical trial in the USA for patients with spinal cord lesions suffering from neurogenic bladder.

2. Inflammation and spinal cord injuries. The unit demonstrated the dysregulation of the macrophage Nrf2 signaling pathway in the chronic neuroinflammatory process observed after spinal cord injuries. The macrophages drive also the heterotopic ossifications frequently observed after spinal cord lesions. Repetitive transcranial magnetic stimulations (rMS) can improve the abnormal macrophages functions (Patented by the unit).

3. Technological Innovations in Mobility and Rehabilitation: The third theme is led by the Laboratory of studies in Mechatronics and Mobility (LEMM) including a small company in engineering. This team focuses on the development of wearable robotics and mechatronic devices aimed at preventing musculoskeletal disorders and improving mobility. Their work includes the "Teach'Wear" program, which provides solutions for postural training and rehabilitation. The program is supported by the Army Commissariat and is designed to prevent back disorders. Another key innovation from this team is the development of a 3D platform for sensory stimulation through body movement, designed to increase the inclusion of people with disabilities in therapeutic and recreational environments, especially for wheelchair users.

HISTORIC AND GEOGRAPHICAL LOCATION OF THE UNIT

The unit ENDICAP was created in January 2015 by merging several pre-existing research teams. These teams were already established on the site of the UFR Santé Simone Veil at Montigny-le-Bretonneux, which had been inaugurated in December 2012. Since its creation, the unit has occupied space within this site, specifically designed to support the development of its scientific research. Additional space is provided at the Raymond Poincaré Hospital in Garches, a specialized site for neurological and muscular disabilities. This geographic organization facilitates collaboration between researchers and clinicians, though the distance between sites has presented some challenges for daily interaction. Despite these obstacles, the unit has developed strong synergies between the clinical and research teams.

RESEARCH ENVIRONMENT OF THE UNIT

With regard to the research environment, the ENDICAP UMR1179 unit is integrated into the UVSQ University and is attached to the UFR Santé Simone Veil at Montigny-le-Bretonneux. The unit operates within a research field focused on neuromuscular and neurological biotherapies, with its main themes spanning gene therapy, rehabilitation technologies, and translational medicine. At the institution level, the unit is part of a larger research

ecosystem that includes both clinical and preclinical research teams working in collaboration with Raymond Poincaré Hospital. This Hospital, located in Garches, specializes in the care of patients with severe neurological and muscular disabilities, providing the unit with access to patient cohorts and clinical expertise crucial for translational research. The unit is a member of various research federations, including the FHU Phénix and the IHU-B HandiMedEx, which are dedicated to innovations in the medical field, particularly for neurological handicaps. Additionally, the unit has contributed to creating a regional innovation network, bringing together researchers, clinicians, and industry partners such as SQY Therapeutics and a biotech spinoff from the unit. The involvement of these regional actors enhances the translational potential of the research conducted at the unit. The unit is also involved in nationally recognized structures, including programs supported by the PIA (Programme Investissements d'Avenir), such as LabEx and Equipex projects. These initiatives aim to support groundbreaking research and the development of high-tech platforms for clinical applications. At the regional level, the unit participates in several regional clusters focused on health and biotechnology (FHU, IHU, ISTY, LEMM), ensuring its active role in the Île-de-France innovation ecosystem. This involvement supports the unit's access to cutting-edge technologies and fosters collaborations with industry partners and other academic institutions. Furthermore, the unit is significantly involved in platforms with both national and international reach. It takes advantage of the local technical platforms performing imaging, genomics, mass spectrometry, and preclinical animal experimentation. These platforms provide the necessary infrastructure for conducting high-impact research and advancing therapeutic innovations. For example, the unit collaborates with platforms for preclinical testing of gene therapies, which are essential for advancing treatments to clinical trial stages.

This comprehensive network of partnerships and access to state-of-the-art facilities enhances the unit's ability to conduct translational research, from basic science discoveries to therapeutic applications.

UNIT WORKFORCE: in physical persons at 31/12/2023

Catégories de personnel	Effectifs
Professeurs et assimilés	19
Maitres de conférences et assimilés	6
Directeurs de recherche et assimilés	3
Chargés de recherche et assimilés	2
Personnels d'appui à la recherche	5
Sous-total personnels permanents en activité	**35**
Enseignants-chercheurs et chercheurs non permanents et assimilés	14
Personnels d'appui non permanents	8
Post-doctorants	1
Doctorants	17
Sous-total personnels non permanents en activité	**40**
Total personnels	**75**

DISTRIBUTION OF THE UNIT'S PERMANENTS BY EMPLOYER: in physical persons at 31/12/2023. Non-tutorship employers are grouped under the heading "others".

Nom de l'employeur	EC	C	PAR
UVSQ	23	1	
Inserm	0	2	
Autres	2	2	
Total personnels	**25**	**5**	**5**

GLOBAL ASSESSMENT

Overall, the unit is excellent. It is recognized internationally because it characterized (i) new type of oligo-antisens (tricycloDNA) and viral vectors now in clinics for neurological and muscular diseases, (ii) the role of inflammation in spinal cord injuries complications (3 patents) and (iii) new technological solutions for the prevention and compensation of motor handicaps. The unit produced 483 articles (50% scientific, versus 50% clinical papers) including 145 as leading authors and on renown journals such as Cells and NAR and gathered 5.5 M€ during the evaluated contract, sometimes from highly competitive academic (5 ANR, 2 European projects, including 4 as PI) or charities (30 contracts, including AFM-Telethon strategic projects mainly as PI). The unit is attractive as 3 additional PIs joined the unit and 10 postdocs from abroad as well as 57 PhD from 10 different French universities were trained. The unit had strong links with society, in particular with lay organizations to disseminate knowledge such as patient's associations (5), HAS recommendations (6) and with private companies such as Renault, Carta-Rouxel or startups such as SQY therapeutics, or EG427 created from the unit itself. The unit suffers from the low number of permanent researchers according to the diversity of their research activities, but their strong translational approach allowed to focus on patient's solutions with large financial outsources.

DETAILED EVALUATION OF THE UNIT

A - CONSIDERATION OF THE RECOMMENDATIONS IN THE PREVIOUS REPORT

According to the 2019 recommendations, the unit answered positively all queries and in particular demonstrated its capacities
- to bring different projects at an industrial level (SQY Therapeutics; EG427; Teachwear TWIZNCAP).
- to increase the number of training towards handicap for scientists at the Bachelor as well as Master level. The unit recently obtained a European MSCA Doctoral Network
- to improve its visibility at the international level using a unique affiliation, participating to EU projects (work package leader in the BIND H2020 project; PI of an ERA NET Neuron grant) and acquiring an international recognition as participants of two COST Actions.
- to support the clinical research of the Raymond Poincaré University Hospital for patients with neurological or neuromuscular handicaps.

A point of attention remains the low number of full-time researchers (EPST) in comparison with the high number of teaching researchers (EC) (ie: 5 versus 25). The unit has tried to obtain an Inserm position for one of their candidates without success. However, the UVSQ continues to support the unit with a new University position to come. They have a continuous recruitment of post-doctoral fellows, the best of them is employed by the associated spin-off or industrial companies, partners of the unit.

B - EVALUATION AREAS

Guidelines for all areas of evaluation (1, 2, 3 and 4): Considering the references defined in the unit's evaluation guidelines, the committee ensures that a distinction is made on the outstanding elements for strengths or weaknesses. Each point is documented by observable facts including the elements from the portfolio. The committee assesses if the unit's results are consistent with its activity profile.

EVALUATION AREA 1: PROFILE, RESOURCES AND ORGANISATION OF THE UNIT

Assessment on the scientific objectives of the unit

The unit's overall strategy is **excellent**, combining f**undamental scientific work** to identify **therapeutic targets** (e.g. teams 1 and 2) or develop **technical tools** (e.g. teams 2 and 3), with **translational work** in collaboration with the **clinical departments of the Raymond Poincaré University Hospita**l and partnerships with **biotechnology companies** such as SQY Therapeutics, EG427, Carta-Rouxel.
This approach facilitates the transition from laboratory research to clinical applications, ensuring the **development of innovative treatments and technologies** for neuromuscular and neuromotor diseases.

Assessment on the unit's resources

The unit is a large research center expanding since 2018 with 3 additional PI from recruitments or joining teams (Teams 1 and 3), which is very good in terms of human resources. The proportion of permanent researchers vs. permanent technical staff is low with **30 permanent researchers** (25 EC, then 17 equivalent full-time) with only **5 permanent PAR.** In addition, the number of full time EPST researchers remains low (5). On the other end, the level of funding is outstanding, amounting to **5.5 M€ with an average of 922 k€ per year** obtained from national sources, such as Inserm and UVSQ, and international sources like H2020 and BpiFrance. Overall, unit **ressources are excellent.**

Assessment on the functioning of the unit

The functioning of the unit is mainly driven by the unit director. Very few formal scientific or strategic meetings exist in order to stimulate interactions within the teams, optimized human resources and clinical translation. The **cohesion of the unit exists** but is mainly based on the **strong and open leadership of its director**.

1/ The unit has set itself relevant scientific objectives.

Strengths and possibilities linked to the context

The three teams have **complementary approaches** and demonstrated their capacities to bring their research to clinic: 1. Team 1 optimized innovative gene therapies. They developed original tricyclo-DNA conjugated to palmitic acid in order to improve the delivery of antisense oligonucleotides to the muscles for exon 51 skipping therapeutic strategies in DMD. A clinical trial has been initiated in 2023 by SQY Therapeutics. In addition, they demonstrated the delivery to the CNS also affected in DMD. In parallel, another group of the team demonstrated the interest of Herpes virus 1 vector for targeting the sensory neurons of the bladder to treat the dysfunctions observed after spinal cord lesions. A company EG427 have been created for translation to patients, 2. Team 2: In spinal cord injuries, Team 2 demonstrated the role of Nrf2 inhibition in the chronic inflammation observed. They developed patented therapeutic strategies using Nrf2 activation in human macrophage by Nrf2 activators or repetitive magnetic stimulation, 3. Team 3 used different technologies to improve motor and respiratory handicaps. In collaboration with the CARTA-ROUXEL SME, they optimized instrumented clothes to detect bad position at work in order to prevent musculoskeletal dysfunctions. A new technology is developed with RENAULT in order to avoid transfer from wheelchair to car. Three different technologies (SEEM; Actimetrie; ventilatory support) have been tested (5 clinical projects) and a web platform developed for home monitoring or reeducation (3 theses).

Weaknesses and risks linked to the context

The range of skills and platforms required is very broad: from muscle gene therapy to spinal cord injuries and technologies for monitoring and reeducation of various handicap. Each team is specialized and for this reason, **insufficient interactions exist between teams** limiting the human resources for each team. Transversal activities and projects are lacking in order to support the most innovative approaches.

2/ The unit has resources that are suited to its activity profile and research environment and mobilises them.

Strengths and possibilities linked to the context

The unit has a great dynamism with strong financial supports due its very successful translational approach (ANRs, EU H2020, Start up, SME). Post-docs are largely supported by the patient associations, start up or SME. In addition, the PhDs are often supported by patient's associations. The number of ANR (n=5, including 4 as PI) and PHRC (n=3, including 2 as PI) obtained, demonstrates the quality of the unit's work.

Weaknesses and risks linked to the context

There is an imbalance between researchers and teacher-researchers (only 5 researchers). A large number of teacher-researchers are also clinicians. This situation is an advantage for translational activities but is a weakness in term of research prospective and management. Collaborations and resources from large European or international projects are lacking perhaps related to the small number of full researchers. The geographical remoteness of the various structures and the atomization of structures with multiple supervisory bodies (IHU, CHU, UMR...) can be a source of governance difficulties.

3/ The unit's practices comply with the rules and directives laid down by its supervisory bodies in terms of human resources management, safety, environment, ethical protocols and protection of data and scientific heritage.

Strengths and possibilities linked to the context

The unit has multidisciplinary and translational approaches. This translational concern induces the need for strong ethical protocols and protection of data at the preclinical and clinical level, which is well managed by the unit members. Human resources management is mainly based on informal exchanges within the director and the team leaders, with success as far as the committee was able to judge.

Weaknesses and risks linked to the context

Interactions between teams could be improved by more formal exchanges at the scientific and management level (regular scientific meetings, unit council with circulating minutes…)

EVALUATION AREA 2: ATTRACTIVENESS

Assessment on the attractiveness of the unit

The unit has implemented an **excellent policy** for stable recruitment of junior researchers (57 PhD; 10 post-Doc fellows hired). Members are involved in international clinical trials/start up (SQY therapeutics, EG427), editorial committees (e.g. board member in the Oligonucleotide Therapeutics Society) and international EU projects (e.g. work package leader in the H2020 BIND project). These actions enhance the unit's visibility, establishing it as an internationally renowned center for neuromuscular and neurological disease therapies.

1/ The unit has an attractive scientific reputation and is part of the European research area.

2/ The unit is attractive because for the quality of its staff support policy.

3/ The unit is attractive through its success in competitive calls for projects.

4/ The unit is attractive for the quality of its major equipment and technical skills.

Strengths and possibilities linked to the context for the four references above

The unit has an excellent level of publications and participation to congress attracting PhD from other French Universities. The unit is successful for competitive calls (ANR; PHRC/PHRI; AFM-Telethon…). The quality of its translational research in the field of motor handicap is unique due to the proximity of the Raymond Poincare Hospital, attracting clinicians but also scientists. The technological platform and the capacity to collect a large number of patients involved in the research is also a key aspect of their attractiveness.

Weaknesses and risks linked to the context for the four references above

Among the 57 PhDs, only 5 are foreigners. The number of Post-Doc (10) remains low given their visibility, but the pandemic may have altered their recruitment slightly (only one at present). The number of international proposals remains low with only one H2020 BIND project as work package leader and one ERANET-Neuron as PI.

EVALUATION AREA 3: SCIENTIFIC PRODUCTION

Assessment on the scientific production of the unit

Scientific output is high in terms of **number of publications (n= 483,** including 145 as leading authors). There are also publications in renown journals in the discipline, without however reaching the "outstanding" level. **This is then overall excellent.** Of particular note is a publication in nucleic acid research (Relizani et al. 2022) on the improvement of tricyclo-DNA splice switching oligonucleotides for the treatment of DMD patients.

1/ The scientific production of the unit meets quality criteria.

2/ The unit's scientific production is proportionate to its research potential and properly shared out between its personnel.

3/ The scientific production of the unit complies with the principles of research integrity, ethics and open science. It complies with the directives applicable in this field.

Strengths and possibilities linked to the context for the three references above

The laboratory's strength is first **quantitative (483 publications; 23 book chapters)**, with significant leadership since 1/3 of them are published with first or last authors from the unit. The other strength on this aspect is the quality of the publications, some in high-impact or important impact journals in the discipline, without however reaching the "outstanding" level. Of particular note is a publication in Nucleic Acid Research (Relizani et al., 2022), which reports the improvement of the muscular delivery of antisense oligonucleotides using palmitic acid conjugation and its interest in DMD therapeutics. Most articles are linked to scientific research, including clinical research activities, while 16 are pure clinical reports. All these aspects are demonstrating the strong involvement of the unit members in a highly visible research activity in this laboratory.

Weaknesses and risks linked to the context for the three references above

Only Team 1 publishes a significant number of PDC articles, including both researchers and students. The situation is more unbalanced for the other teams. There are many publications with one or two team members, one of whom is often in the middle. Some only publish in the middle of the list without a PDC. There are also some non-publishing or low-publishing team members. And what's most striking is the **low number of publications including different members of the unit and also of the same team**. So, the overall impression is one of a high level of production and motivation to publish, with very limited internal collaboration, including between members of the same team. The evaluation committee takes note of the need for **teacher-researchers** to be attached to accredited research teams, and of the difficulties this may entail for a team developing a targeted project. It is necessary to strike a balance between institutional and local constraints and the conduct of a scientific project. It would seem advisable to try and find ways of pooling and leveraging resources around research projects. Participation and communication at scientific conferences are of high quality. Invitations to prestigious conferences may be lacking (only 5 international conferences).

EVALUATION AREA 4: CONTRIBUTION OF RESEARCH ACTIVITIES TO SOCIETY

Assessment on the inclusion of the unit's research in society

Dissemination activities are of **high quality**. Laboratory members are involved in a wide range of teaching activities, both on and off campus. They help to disseminate knowledge at regional level through events organized by their universities. Some are also involved in public communications, whether on radio or television, or through associations in correlation with their translationnal activities but also as researcher. Collaborations with SME and startup are also important. **The committee believes these facts indicate an outstanding interaction with the society.**

1/ The unit stands out for the quality and the amount of its interactions with the non-academic world.

2/ The unit develops products for the cultural, economic and social world.

3/ The unit shares its knowledge with the general public and takes part in debates in society.

Strengths and possibilities linked to the context for the three references above

The unit, by its translational activity, develops by definition **large contacts with the economic and social world** particularly in the domain of handicap (recommendations for 9 diseases and for ventilation for intensive care unit, development of new technologies for rehabilitation such as TWIZNCAP, Home Monitoring). The unit has strong interactions with **patient' associations** (AFM-Telethon, Spinal injuries association, SQY therapeutics has been created by 2 patient associations: Duchenne Parent Project & Association Monégasque contre les Myopathies) and the general public (fetes de la science, participation to TV show, etc). Collaborations with CARTA-ROUXEL SME and Renault for example are illustrations of their interaction with private companies. Members of the unit also participated to the creation of startups, SQY Therapeutics and EG427. In addition, 5 patents have been submitted by the unit and team 3 created the first Labcom of the UVSQ.

Weaknesses and risks linked to the context for the three references above

Apart from one member of the laboratory, there are **no responsibilities in national or international** learned societies. There is, however, participation in the organization of congresses (15) and HAS-type evaluation committees (5) by other PIs.

ANALYSIS OF THE UNIT'S TRAJECTORY

During the last 10 years (2015-2025), the unit brought together local forces able to realize the site project initiated by IHU-B HandiMedEx (2011-2016) and contributed significantly to the creation of the FHU Phénix (2020-2024). This project brought together and mobilized a set of multidisciplinary teams of researchers and doctors working in the field of myopathies, the pathophysiology of spinal cord injuries, medical complications associated with certain locomotor deficits, and the search for new medical devices for the prevention of musculoskeletal disorders and mobility. The health UFR, patient associations as well as SQY Therapeutics, and startup has play an important role in the success of the unit. The development of medical devices for disability and mobility have contributed to forging a strong identity of the UVSQ in terms of innovative research for the treatment of motor disabilities. Dissolution of the actual unit is proposed into 3 entities:
1/ **IMPROVE** (INSERM-UVSQ Research unit labelling) formed by people from teams 1 and 2 of the ENDICAP unit and the team RHuMA of the UMR 1198 – BREED. IMPROVE will develop fundamental and preclinical researches in the field of neuromuscular disorders and gene therapy approaches for erectile dysfunctions after spinal cord injuries (mainly people coming from team 1 ENDICAP) and reproduction (RHUMA team). The structure into a single team would be more favorable in the beginning, in order to strengthen the links between the two planned teams.
2/ **PHARMAColigo** (UVSQ Research unit labelling) formed by people from teams 1, 2 and 3 of the ENDICAP unit. This unit will develop a translational research aiming to characterized new routes of administration for ASO-tcDNA, to identify new biomarkers for clinical evaluation (safety & efficacy), to design new clinical protocols for the after AVANCE 1 trial and further lead compounds that are under development. This new unit will form the University entity with which SQY Therapeutics will collaborate.
3/ **REHADAPT**. Team 3 of ENDICAP will be distributed over PHARMACOLIGO and the existing ERPHAN (UVSQ labelled Research unit since 2020), which will become REHADAPT in 2026. REHADAPT will optimize devices and web platforms for evaluation and readaptation of motricity and ventilation in neurological and neuromuscular disorders with an APHP label. It will be associated with the Institut en santé de para-sport connecté (ISPC).

This restructuration seems logical given the different approaches of each team. These entities will be associated with the nonprofit SQY company in an informal structure named Ecosystème Hospitalo-Universitaire. The functioning of this structure, including private companies, UVSQ, Paris Saclay Université, Inserm, CNRS, CEA, APHP entities is not well defined yet, however. The creation of a foundation including the different entities with the strong support of the associated patient's associations will be perhaps more adapted.

RECOMMENDATIONS TO THE UNIT

Recommendations regarding the Evaluation Area 1: Profile, Resources and Organisation of the Unit

The unit is multidisciplinary with **excellent and successful translational skills** leading to an impressive institutional resources. There is however an imbalance between researchers and teacher-researchers. The evaluation committee notes the difficulty of recruiting researchers on a permanent basis, due to the low number of positions available at national level. In this case, it would be desirable for some of the teacher-researchers to be relieved of their teaching and clinical duties, so that they can devote themselves fully to research, either in the form of an interface contract, or possibly by applying to the Institut Universitaire de France. As for the rest of the staff, the unit is relatively rich in technical personnel provided by the University and poorer in statutory personnel provided by Inserm (5 permanent versus 8 non-permanent people). Interactions between teams and future units must be developed through transversal activities (clinical research; common groups of patients…) and more formal regular meetings at the unit level (seminars…). The cohesion of the unit is due to the leadership of his director. More formal management of the unit must be developed.

Recommendations regarding the Evaluation Area 2: Attractiveness

The unit attracted a large number of PhD students and clinicians as well as teachers interesting in translational research. The large number of publications and the originality of the projects are strength for attractiveness. However, a **larger involvement in European and international projects** is needed in order to attract more foreign post-docs or PhDs and to optimize technological issues.

Recommendations regarding Evaluation Area 3: Scientific Production

The unit has a large scientific productions in terms of original articles, patents and clinical researches. Participation to international collaborative projects will probably **increase the number of publications in journals with larger readership.**

Recommendations regarding Evaluation Area 4: Contribution of Research Activities to Society

The research activity being mainly translational, contribution of research activities to society is significant. The unit must develop a more active politic of dissemination by using **webinars, podcasts, web unit, and through actors of health care such as APHP/Inserm, as the unit has the recognition on therapeutics in neuromuscular diseases.**

TEAM-BY-TEAM ASSESSMENT

Team 1: Biotherapies for Neuromuscular Diseases (future evolution into 2 units, IMPROVE and PHARMAColigo)

Name of the supervisor: Ms Aurélie Goyenvalle

THEMES OF THE TEAM

The Biotherapies for Neuromuscular Diseases team (team 1) focuses on the development of **antisense or gene therapy for neurological and neuromuscular diseases.** Its activity follows three main themes: 1- optimisation of **tricycloDNA** for the treatment of Duchenne Muscular Dystrophy (DMD); 2 -physiopathology of **muscular dystrophies** to find new therapeutic targets; 3- use of recombinant **Herpes Simplex Viruses 1 for gene therapy** in neurological uro-genital dysfunctions following spinal cord injuries.

CONSIDERATION OF THE RECOMMENDATIONS OF THE PREVIOUS REPORT

The **large majority of recommendations have been followed and are summarized below:**
- Common unit membership for scientific publications now exists;
- Visibility in the media was largely increased;
- Publications between researchers and clinicians are now frequent;
- 2 Cifre fellowships have been obtained;
- translation of research into clinical trials have been initiated with SQY and EG427.

However, there are still few points that need to be improved:
- The number of foreign post docs and international projects remain low;
- Medical students have been included in master or PhD program. However, formal meetings to discuss the signification of fundamental research for clinic problems, the design of preclinical trials, the natural history of future clinical translation, the design of clinical trials or bibliography analysis are still lacking;
- Many HDR holders, mainly clinicians included in the team, are not supervising PhD students but this situation is frequent in other research units;
- The role of the team leader to assure links between the neuromuscular and the neurogenic uro-genital group is still not clearly defined.

WORKFORCE OF THE TEAM: in physical persons at 31/12/2023

Catégories de personnel	Effectifs
Professeurs et assimilés	9
Maitres de conférences et assimilés	2
Directeurs de recherche et assimilés	2
Chargés de recherche et assimilés	1
Personnels d'appui à la recherche	7
Sous-total personnels permanents en activité	**21**
Enseignants-chercheurs et chercheurs non permanents et assimilés	5
Personnels d'appui non permanents	2
Post-doctorants	1
Doctorants	8
Sous-total personnels non permanents en activité	**16**
Total personnels	**37**

EVALUATION

Overall assessment of the team

Team 1 was very successful in the development of innovative therapies for neurological and muscular disorders with **two products in phase 1-2 clinical trials**. A first group focused their research on the development of **exon skipping antisense therapy for Duchenne Muscular Disorder (DMD) using TricycloDNA**. To this end, in close collaboration with SQY Therapeutics, they are progressively optimising tricycloDNA in terms of targeting, pharmacology and toxicity using an innovative palmitoyl acid conjugation in strong collaboration with SQY therapeutics. A preclinical proof of concept was obtained in 2022 for the skipping of exon 51 (patent). The permanent interfaces with clinicians and patients' associations involved in DMD allowed the rapid application for clinical trial in 2023 on the Raymond Poincaré hospital site. The second group used the **high affinity of the herpes virus type 1 for the sensory neurons** to develop an in vivo gene therapy to modulate the hyperactive muscle bladder sphincter observed after spinal cord injuries. A patent and the creation of a start-up (EG427) in 2022 has led to a clinical trial in USA in 2024. The production (257 scientific articles, ~3 articles per year per PI), 1/3 as leading authors and 50% as pure scientific papers (vs clinical), is excellent.
Overall, this is an excellent to outstanding team.

Strengths and possibilities linked to the context

The team published 257 scientific publications, including 75 as first or last-author, in excellent speciality journals as Nucleic Acid Research, PNAS or Cells. Staff resources include 3 Full-time researchers (DR and CR), 9 part-time medical researchers and 7 permanent technical staff. The high financial resources (4.4 M€ over 5 years) has been provided by various supports: 2 EU (H2020-BIND and ERANET as WP leader or PI, 4 ANR as PI, 1 SATT as PI and 20 projects from 5 different Charities as PI. The team has an international visibility due to its participation to International networks (2 COST Actions, BIND & TACT). PIs participated to more than 30 jurys for PhD defence or HDR. Their excellent international recognition for tricycloantisens and HSV1vectors is evident but also for neuromuscular disorders (DMD) and genito-vesico sphincter problems in spine cord injuries. The PIs show excellent translational capacities with high quality of the fundamental and preclinical research in strong contact with clinicians and patients' associations and leadership for the creation of SQYtherapeutics with the financial support of a charity and of EG427 with the Paris-Saclay SATT support. The team also submitted 5 patents. They also have strong interactions with the family associations and frequent participation (each year) to TV shows, public information on sciences, etc. They also participated to four HAS recommendations.

Weaknesses and risks linked to the context

The number of full-time researchers (3) is low for the daily management of PhD students and technical staff. In addition, only 2 Post docs have been mentioned despite the international recognition of the team. No links between the main 2 research themes of the team exits from a scientific point of view. The percentage of joint publications between physicians and biologists must be optimized by the development of more formal weekly or monthly scientific meetings.

Analysis of the team's trajectory

Team 1 has the **leadership of the unit since its creation in 2015**. Its trajectory is **exemplary** with the development of innovative tricycloDNA for therapeutic use in one of the most frequent myopathy, the DMD. During the 2018-2023 period, the team has stimulated the platforms and facilities, which support research and facilitate the transition to clinical research. The team ensures that **scientific advances are rapidly transformed into therapeutic applications**. The creation of spin-off companies such as SQY Therapeutics and EG427 demonstrates the **ability of the team to translate its research into concrete clinical solutions,** offering hope to patients with severe neuromuscular and neurological disorders. The team has maintained an excellent innovative research on the use of tricycloDNA for the CNS or in combination with other therapeutic approaches but also for erectile dysfunctions after spinal cord injuries. After these remarkable successes, the new prospectives are challenging. The proposition for the future is to **create 2 new research units, IMPROVE** (USVQ-Inserm) **and PHARMAColigo** (UVSQ), from members of this team and of team 2.

A/The future "IMPROVE" unit will include 2 teams:

-The **team biotherapies for Neuromuscular Diseases** will be an evolution of part of the present teams 1 and 2. This new team will have 2 main objectives. The first one will pursue the development of tricycloDNA for therapy by (i) targeting the CNS using as a proof of concept cognitive dysfunctions related to dystrophinopathies and

(ii) improving their biodistribution. The second objective will pursue the therapeutic approaches of spinal cord injuries by (i) developing the HSV1 gene therapy for erectile dysfunctions and (ii) including a new team for improving the ventilatory dysfunctions using rMS and magnetogenetic. How the project on spinal cord injuries will be implemented, on which aspects, is still puzzling.
-The second team is coming from the team **RHuMA** of the UMR 1198 – BREED, already in the same UVSQ site. This team has a research interest in the **field of infertility.** The joint with IMPROVE is explained by the development of innovative therapies for (i) the alterations of the gametogenesis, (ii) the infertility related to anticancer drugs and (iii) the extra-uterine adaptation of the neonate**.** The team RHuMA (future IMPROVE unit team) demonstrates strong scientific achievements, impactful research, and effective training. Challenges exist in resource balance and team stability. In addition, the proposed therapeutic strategies did not match with the know-how of members of team 1 joining IMPROVE. No complementary platforms or clinicians network seems to emerge between the 2 future IMPROVE teams. The communities are wondering whether a joint team should be set up from the outset to strengthen each theme through synergies?

B/The future unit "PHARMAColigo" will be an evolution of parts of the present team 1 and team 2 of ENDICAP. The objectives of this new unit will be to develop a translational research (from fundamental to applied), in collaboration with SQY Therapeutics, aiming to characterize new routes of administration for ASO-tcDNA, to identify new biomarkers for clinical evaluation (safety & efficacy), and to design new clinical protocols for further lead compounds that are under development. However, the new PHARMAColigo entity will include only **UVSQ** as label. The project as presented is dedicated to the clinical development of the SQY products and to clinical research (biomarkers, etc), but will be separated from the more fundamental work done by the future IMPROVE unit. As presented, one major risk is the potential toxicity associated with the tcDNA chemistry used in the current clinical research, which could lead to its clinical development being halted. Although no adverse effects have been documented to date for the SQY51 drug candidate, the long-term effects on humans remain unknown, and the clinical benefit to patients can only be determined once the current trial phase has been completed. The production of tcDNA and ASO-tcDNA could be also a challenge for clinical development. Finally, the administration of the Hospital is actively seeking alternative locations to accommodate future clinical units, indicating an urgent need for new facilities to support the proposed research effectively.

RECOMMENDATIONS TO THE TEAM

The evolution of team 1 **aligns well with the previous success**, **the number of researchers, their clinical expertise and their international interactions** making it a sound decision. The use of tricycloDNA can be a challenge due to the monthly IV injection needed, to unexpected long-term toxicity as described for other antisense strategies in balance with the development of full length dystrophin gene therapy using new safer AAV vectors after a single IV injection. The development of magnetogenetic using an AAV with an electromagnetic promotor stimulated by rMS for modification of the local environment after spinal cord injury is innovative but could be challenging in vivo.
In the future PHARMAColigo unit, the project is mainly devoted to translational research and clinical application to SQY products. The sustainability of the project in case of difficulties in the clinical development of SQY products (toxicity but also new innovative therapies for DMD patients) is not mentioned. The inclusion of the project in a **sustainable clinical research entity must take into account the future of the clinical site of Raymond Poincaré Hospital.**

Team 2:	Disability and Inflammation (future evolution into 2 units, IMPROVE and PHARMAColigo))
Name of the supervisor:	Mr Marcel Bonay

THEMES OF THE TEAM

The Disability and Inflammation team (**team 2),** focuses on key themes related to spinal cord injuries. They explore the **physiopathology of complications,** especially **respiratory insufficiency** and **bone issues**, following such injuries. A major focus is on **understanding the role of inflammation** in these complications, emphasizing the importance of managing immune responses. The team also investigates the therapeutic potential of **physical exercise** in improving functional and respiratory recovery. Additionally, they study the **neurophysiological effects of repetitive magnetic stimulation**, particularly its anti-inflammatory and antioxidant properties. They aim to develop **innovative therapeutic strategies** for spinal cord injury patients, contributing to both **translational research** and clinical applications.

CONSIDERATION OF THE RECOMMENDATIONS OF THE PREVIOUS REPORT

Team 2 has made significant progress by following the recommendations of the previous committee:

1. Human Resources:

The team was encouraged to address age structure issues by recruiting mid-career researchers, particularly permanent staff, to balance the age pyramid. They were also advised to recruit technical and administrative staff to support the sustainability of developed technologies and expertise. The team has successfully recruited several PhD students and post-docs, including international candidates, addressing earlier recommendations on attracting fresh talent. However, permanent mid-career recruitment remains a challenge.

2. Research Focus:

Team 2 was encouraged to broaden its focus and explore innovative therapeutic strategies for addressing respiratory insufficiency and bone complications from spinal cord injuries. The team continues to work on the physiopathology of spinal cord injuries, focusing on inflammation and respiratory insufficiency, with strong emphasis on repetitive magnetic stimulation (rMS) and exercise therapy.

3. Collaborations and Technological Advancements:

The recommendations emphasized strengthening technological platforms and maintaining international collaborations to enhance the unit's impact and visibility. Involvement in international collaborations and participation in editorial committees has enhanced the team's scientific reputation, fulfilling previous recommendations.

In conclusion, **team 2 has addressed several prior recommendations,** especially regarding technological advancements and research expansion, though permanent staff recruitment still requires attention.

WORKFORCE OF THE TEAM: in physical persons at 31/12/2023

Catégories de personnel	Effectifs
Professeurs et assimilés	4
Maitres de conférences et assimilés	2
Directeurs de recherche et assimilés	1
Chargés de recherche et assimilés	1
Personnels d'appui à la recherche	1
Sous-total personnels permanents en activité	**9**
Enseignants-chercheurs et chercheurs non permanents et assimilés	7
Personnels d'appui non permanents	0
Post-doctorants	0
Doctorants	5
Sous-total personnels non permanents en activité	**12**
Total personnels	**21**

EVALUATION

Overall assessment of the team

Team 2, has established a strong reputation in spinal cord injury (SCI) research, focusing on the **physiopathology of respiratory insufficiency** and inflammation. Their innovative therapeutic strategies, like **repetitive magnetic stimulation (rMS)** and exercise therapy, reflect a commitment to improving patient outcomes. Among their main achievements, one could note the modulation of NRF2 in macrophages by in vivo stimulation to increase their efficacy. While the team has successfully recruited PhD students and post-docs, attention is needed for permanent mid-career recruitment to balance the age structure.
Overall, this is a very good to excellent team that produced 92 research articles (1-2 article per year per PI).

Strengths and possibilities linked to the context

Team 2, has established a **robust reputation in the field of spinal cord injury research**, particularly for its focus on innovative therapeutic strategies that address critical issues such as **respiratory insufficiency and bone complications**. The team has made significant strides in developing novel interventions, notably repetitive magnetic stimulation, which presents a non-invasive treatment option with the potential to enhance recovery for individuals with spinal cord injuries. Their commitment to exploring a range of therapeutic approaches demonstrates a proactive stance towards addressing complex health challenges. Moreover, team 2 has forged strong collaborations with Raymond Poincare Hospital (intensive care, orthopaedic/rehabilitation units) **facilitating the translation of findings into clinical applications**. These partnerships provide valuable resources and support for clinical trials. The integration of multidisciplinary expertise within the team fosters an environment conducive to innovation. Team 2 is committed to **scientific dissemination and public engagement**. Their participation in various conferences, workshops, and community events reflects their dedication to sharing knowledge and raising awareness about spinal cord injuries and related complications. Lastly, the team's **strong socio-cultural connections** with patient associations underscore their commitment to patient-centered research.

Overall, these strengths provide a solid foundation for Team 2's future endeavours and highlight their potential for significant contributions to both research and societal well-being.

Weaknesses and risks linked to the context

Despite Team 2's strengths, several weaknesses and risks could hinder its progress. **Additional preclinical trials** and related physiopathological studies are needed in order to obtain more convincing proof of concepts for clinical applications. There are only 2 scientific publications on the NRF2 subproject since 2020 and the underlying mechanisms of repetitive stimulation still remains to be understood. The number of **full-time researchers** (2) and permanent technical staff (only one) seems **low** compare to the objectives. The remaining 6 permanent staff resources are part time in teaching and/or clinics. A total of 800,000 euros over 5 years (ANR, SATT, Associations) of grants seems insufficient to meet the stated objectives. According to this low number of researchers and resources, the team will have interest to **focus on one therapeutic approach in one model of spinal cord injuries**.
Team 2 also lacks international appeal.

Analysis of the team's trajectory

Team 2, "Handicap et Inflammation," has seen a **positive trajectory** since its inception within the unit. Established in 2015, the team has consistently aligned its activities with the objectives outlined at its creation. The team has actively participated in the development of a collaborative ecosystem, contributing to significant projects like the IHU-B HandiMedEx and the FHU Phénix. This ecosystem comprises multidisciplinary teams of researchers and medical professionals focused on understanding myopathies and the physiopathology of spinal cord injuries. As part of a significant restructuring initiative, the team is set to **evolve into new entities** that will maintain the focus on improving care for incurable neuromuscular diseases. The proposed changes, including a restructuring into **PHARMAColigo**, will enhance the **team's clinical research capacity** and ensure its leading position in developing innovative therapies. Overall, **team 2's trajectory reflects its commitment to advancing research and clinical practices, with an ongoing focus on societal impact and patient care.**

The proposition for the future is to **create 2 new research units, IMPROVE** (USVQ-Inserm) **and PHARMAColigo** (UVSQ), from members of this team and of team 1.

A/The future "IMPROVE" unit will include 2 teams:
-The **team biotherapies for Neuromuscular Diseases** will be an evolution of part of the present teams 1 and 2. This new team will have 2 main objectives. The first one will pursue the development of tricycloDNA for therapy by (i) targeting the CNS using as a proof of concept cognitive dysfunctions related to dystrophinopathies and (ii) improving their biodistribution. The second objective will pursue the therapeutic approaches of spinal cord injuries by (i) developing the HSV1 gene therapy for erectile dysfunctions and (ii) including a new team for improving the ventilatory dysfunctions using rMS and magnetogenetic. How the project on spinal cord injuries will be implemented, on which aspects, is still puzzling.
-The second team is coming from the team **RHuMA** of the UMR 1198 – BREED, already in the same UVSQ site. This team has a research interest in the **field of infertility.** The joint with IMPROVE is explained by the development of innovative therapies for (i) the alterations of the gametogenesis, (ii) the infertility related to anticancer drugs and (iii) the extra-uterine adaptation of the neonate**.** The team RHuMA (future IMPROVE unit team) demonstrates strong scientific achievements, impactful research, and effective training. Challenges exist in resource balance and team stability. In addition, the proposed therapeutic strategies did not match with the know-how of members of team 1 joining IMPROVE. No complementary platforms or clinicians network seems to emerge between the 2 future IMPROVE teams. The communities are wondering whether a joint team should be set up from the outset to strengthen each theme through synergies?

B/The future unit "PHARMAColigo" will be an evolution of parts of the current team 1 and team 2 of ENDICAP. The objectives of this new unit will be to develop a translational research (from basic to applied), in collaboration with SQY Therapeutics, aiming to characterize new routes of administration for ASO-tcDNA, to identify new biomarkers for clinical evaluation (safety & efficacy), and to design new clinical protocols for further lead compounds that are under development. However, the new PHARMAColigo entity will include only **UVSQ** as label. The project as presented is dedicated to the clinical development of the SQY products and to clinical research (biomarkers, etc), but will be separated from the more fundamental work done by the future IMPROVE unit. As presented, one major risk is the potential toxicity associated with the tcDNA chemistry used in the current clinical research, which could lead to its clinical development being halted. Although no adverse effects have been documented to date for the SQY51 drug candidate, the long-term effects on humans remain unknown, and the clinical benefit to patients can only be determined once the current trial phase has been completed. The production of tcDNA and ASO-tcDNA could be also a challenge for clinical development. Finally, the administration of the Hospital is actively seeking alternative locations to accommodate future clinical units, indicating an urgent need for new facilities to support the proposed research effectively.

RECOMMENDATIONS TO THE TEAM

To further strengthen its impact, team 2 should prioritize the **recruitment of permanent mid-career researchers** to enhance team stability and expertise in analysis of the underlying mechanisms. This will help balance the age structure and foster mentorship for junior members. The team should also focus on securing **additional funding to support clinical trials and research initiatives**, ensuring the continuity of innovative projects, particularly those related to spinal cord injuries in the future IMPROVE unit.
Enhancing collaborations with international research institutions can broaden the team's scope and access to diverse resources and patient populations, ultimately improving patient recruitment efforts for clinical studies. Continued engagement with patient associations is vital to align research objectives with the needs of the community, ensuring that outcomes are relevant and impactful.
In the future PHARMAColigo unit, the project is mainly devoted to translational research and clinical application to SQY products. The sustainability of the project in case of difficulties in the clinical development of SQY products (toxicity but also new innovative therapies for DMD patients) is not mentioned. The inclusion of the project in a **sustainable clinical research entity must take into account the future of the clinical site of Raymond Poincaré Hospital.**

Team 3: Technologies Applied to Neuromotor Disorders (future: PHARMAColigo and REHADAPT units)

Name of the supervisor: Mr Nicolas Roche

THEMES OF THE TEAM

The technologies applied to neuromotor disorders team (team 3) has developed 3 main themes: **1. Mecanotronic and Mobility**. Technologies have been developed by the LEM2 LABCOM (ANR) consortium to optimize instrumented clothing to detect poor positions at work and prevent musculoskeletal disorders. A new technology is also being developed with RENAULT prevent transfers from wheelchairs to cars. **2. Respiratory suppliance technology** (PHRC, PHRIP). The idea is to develop wearable devices for ventilatory support evaluation in the hospital and at home. 3. **Virtual reality and gamification.** It is dedicated to the development of a web platform for re-education.

CONSIDERATION OF THE RECOMMENDATIONS OF THE PREVIOUS REPORT

The previous recommendations were to increase the number of researchers holding a HDR and to develop more national and international collaborations.

During the mandate, no full-time researcher have been recruited unfortunately, but one MD/PhD researcher and a full time Professor at the engineer school ENSAM in Paris and at the Institut des sciences et technologies des Yvelines (ISTY) in Mantes, now work for the project in collaboration with 4 clinicians. At the international level, collaborations with Sherbrooke University (CIC 1429) and Belgrade University (LEM2) are mentioned.

WORKFORCE OF THE TEAM: in physical persons at 31/12/2023

Catégories de personnel	Effectifs
Professeurs et assimilés	6
Maitres de conférences et assimilés	2
Directeurs de recherche et assimilés	0
Chargés de recherche et assimilés	0
Personnels d'appui à la recherche	1
Sous-total personnels permanents en activité	**9**
Enseignants-chercheurs et chercheurs non permanents et assimilés	2
Personnels d'appui non permanents	2
Post-doctorants	0
Doctorants	4
Sous-total personnels non permanents en activité	**8**
Total personnels	**17**

EVALUATION

Overall assessment of the team

The scientific objectives are only **technologic.** The first approach was the development of devices included in cloths in order to detect poor positions at work or able to evaluate ventilatory supplies/abnormal movements during sleep or to stimulate the spinal cord. The second main approach was to create a web platform to record the devices but also to develop rehabilitation at home. The team produced 134 publications (2-3 articles per year per PI) including only 11 research publications, the remaining being mainly clinical publications related to no full-time researchers. On the other end, the team had excellent interactions with industry and patient's organizations. **This is overall a very good team.**

Strengths and possibilities linked to the context

The team has created the first Labcom of the UVSQ University, LEM2, through an ANR support with the SME Carta-Rouxel, and also benefited from a contract with Renault, 2 PHRC and on PHRI for a total of €1,165,000 for which they are PI. The team is composed of 9 permanent persons without full-time researcher. The team hosted 5 PhDs thesis that were defended but also numerous Master students through engineer schools. Given its projects, the team has excellent clinical interface with neurologists, rehabilitation and pneumologists (PHRC, PHRIC) providing large cohorts of patients. PI are involved in teaching at master level (neurologic handicap) and participated to seven HAS recommendations. Attractivity for the industry is high (RENAULT, Carta-Roussel), particularly through the LEM2 labcom. Communication for and with the patients' associations are also frequent.

Weaknesses and risks linked to the context

The **large diversity of the topics** with a limited number of researchers (no permanent full-time researcher) is a clear weakness. For many of the technologies mentioned, such as spinal cord stimulation, exoskeleton, etc., the team does not have **international recognition** and more advance teams exist in the field. The team needs to **better define its specificity** in order to focus its research and optimize the limited human resources at its disposal.

Analysis of the team's trajectory

The workforce of team 3 will be spread across the future PHARMAColigo unit and mainly an already existing UVSQ-APHP unit (ERPHAN) that will become the REHADAPT research unit under the same institutional labels.
In REAHDAPT, they will focus on **Reeducation-Rehabilitation**. Team members will limit their themes to motricity, ventilation and occupational-participative therapy in patients with neurological handicap which seems reasonable regarding the staff available. The themes are related to very important clinical problems (motor evaluation in real life, ventilation adaptation, re-education and occupational performance) and must then be supported in the future unit hosting this team.

RECOMMENDATIONS TO THE TEAM

The focus proposed in the evolution of the team is a good decision according to the number of staff. The themes correspond to the clinical expertise involved in the team.

Strong interactions with technological poles are needed within the Paris-Saclay University resources but also at the national and international level in order to choose the best technologies for the corresponding clinical problem.

Integration of the team in the future of Raymond Poincaré University hospital must be clearly defined in link with PHARMAColigo for the development of clinical outcomes.

CONDUCT OF THE INTERVIEWS

Date

Start: 25 November 2024 at 08:30

End: 25 November 2024 at 18:30

Interview conducted: on-site

INTERVIEW SCHEDULE

November 24th
Arrival in Paris
19:15 Evening dinner (only committee members and Hcéres Scientific advisor)

November 25th
Address: UFR Simone Veil santé, Université de Versailles Saint-Quentin en Yvelines
2 Avenue de la source de la Bièvre, 78180 Montigny-le-Bretonneux

8:30-8:50 Welcome coffee (closed-door): Visiting committee with the Hcéres advisor (room salle VIP Rez-de-chaussée)

8h50-10:00 **Presentation of the evaluation process and the unit scientific outputs** (room Amphi 3 Rez-de-jardin)

8:50-9:00 Presentation of the evaluation process to the unit by the Hcéres advisor

9:00-10:00 Presentation of the unit scientific outputs and strategy by the lab director **Luis Garcia** (35' presentation + 25'discussion)

10:00-10:20 Coffee break (room Hall Amphi 3 Rez-de-jardin)

10:20-11:00 Visit of the local facilities and unit

From 11:00 **Meetings with the various categories of staff (closed door)** (room *Amphi 3 Rez-de-jardin*)

11:00 Discussion with PhD students and post-docs
11:30 Discussion with scientists (without team leaders)
12:00 Discussion with engineers, technicians and administrative personnel (in French)
12:30 Discussion with the team leaders

13:00-14:30 Lunch (closed-door with the committee and Hcéres advisor)
Buffet (room Hall Cafeteria Rez-de-chaussée)

14:30-16:00 **Presentation of the research results by group leaders** (room Amphi 3 Rez-de-jardin) (15' presentation, 15' discussion)

14:30 Aurélie Goyenvalle (**VISIO**)
Team 1 - ENDICAP Biotherapies for Neuromuscular Diseases
Future: UMR IMPROVE *(Aurélie Goyenvalle)*

15:00 Marcel Bonay
Team 2 - ENDICAP Disability and Inflammation
Future: PHARMAColigo *(Helge Amthor & Marcel Bonay)*

15:30 Nicolas Roche
Team 3 - ENDICAP Technologies Applied to Neuromotor Disorders
Future: READAPT *(Nicolas Roche)*

16:00-16:30 *Coffee break* (room ***Hall Cafeteria Rez-de-chaussée***)

16:30-17:30 Debriefing (only committee members and Hcéres Scientific advisor) (room ***salle VIP Rez-de-chaussée***)

17:30-18:00	Discussion with the representative of the managing bodies (closed-door) & local representatives (room ***salle VIP Rez-de-chaussée***)
18:00-18:30	Discussion with the director (closed-door) (room ***salle VIP Rez-de-chaussée***)
18:30	End of the visit

PARTICULAR POINT TO BE MENTIONED

The PhD students and postdocs seem generally satisfied, although some express concerns about a supervisor who is not always available, which is regrettable. In addition, the PhD students seem to be unaware of the opportunities offered by training programs and the application procedures for future positions, and there is no clear policy for preparing their oral presentation for competitions. The lack of enough direct supervision is seen as a disadvantage compared to personal mentoring. This is associated with a certain lack of cohesion between all teams, which should be improved in the future units hosting these teams.

GENERAL OBSERVATIONS OF THE SUPERVISORS

UVSQ
université PARIS-SACLAY

Le Président de l'Université de Versailles Saint-Quentin-en-Yvelines

A

Monsieur Stéphane Le Bouler,
Président
Haut Conseil de l'évaluation de la recherche et de l'enseignement supérieur
2 rue Albert Einstein - 75013 PARIS

A Versailles,
Le lundi 03/03/2025

Ref. DER-PUR260024923 - END-ICAP - Handicap neuromusculaire : physiopathologie, biothérapie et pharmacologie appliquées

Objet : Evaluation des unités de recherche – Volet Observation de portée générale

Monsieur le Président,

Nous avons pris connaissance avec le plus grand intérêt du rapport de l'HCERES concernant l'Unité de Recherche (UMR 1179), dénommée « Handicap neuromusculaire : physiopathologie, biothérapie et pharmacologie appliquées (END-ICAP) », portée par M. Luis Garcia, Directeur et M. Marcel Bonay, Directeur Adjoint.

Nous remercions l'HCERES et le comité pour l'efficacité et la qualité de leur travail d'analyse et pour leurs recommandations constructives.

Nous souhaitions, également, réaffirmer notre appui au projet de dissolution-recomposition de END-ICAP. Ce projet de dissolution repose sur la volonté de créer trois nouvelles unités propres possédant chacune leur unité d'action et s'appuyant sur les thématiques des trois équipes composant à ce jour END-ICAP. Nous soulignons la transversalité de ces projets qui intègrent au sein des nouvelles unités, les chercheurs de ENDI-ICAP sans notion d'appartenance aux équipes existantes à ce jour. L'Université apportera son soutien aux trois directeurs portant ces projets d'unités et à leur équipe afin de permettre toute la réussite de ces nouveaux laboratoires.

Nous vous adressons ci-joint les observations et commentaires du porteur de ce projet formulés au regard du rapport de l'HCERES.

Nous vous prions de croire, Monsieur le Président, à l'expression de nos cordiales salutations.

Professeur Loïc Josseran

Président de l'UVSQ

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www.hceres.fr

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