INSTITUTE OF MYOLOGY
CURE THROUGH INNOVATION

Accelerating muscle research and medecine

2024 ANNUAL REPORT

INSTITUTE OF MYOLOGY

A leading patient-centered institute

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Created in 1996 at the instigation of the AFM-Telethon in partnership with four public institutions – AP-HP, Inserm, CEA, and Sorbonne University (Paris VI) – the Institute of Myology brings together nearly 300 experts in muscle and muscle pathologies who have been working for nearly 30 years at the service of muscle science and medicine, known as myology. This international center of expertise coordinates activities related to patient care, including diagnosis, assessment, treatment, basic and applied research, clinical research, and teaching.

Our priority: Innovate to cure.

Our ambition: To find treatments for neuromuscular disorders.

Our mission: To deepen our understanding of muscle dysfunction in order to develop therapeutic approaches to better treat and support patients.

Our values: Innovation, Commitment, Expertise, Collaboration, Integrity, Knowledge Sharing.

300
muscle experts

SUMMARY

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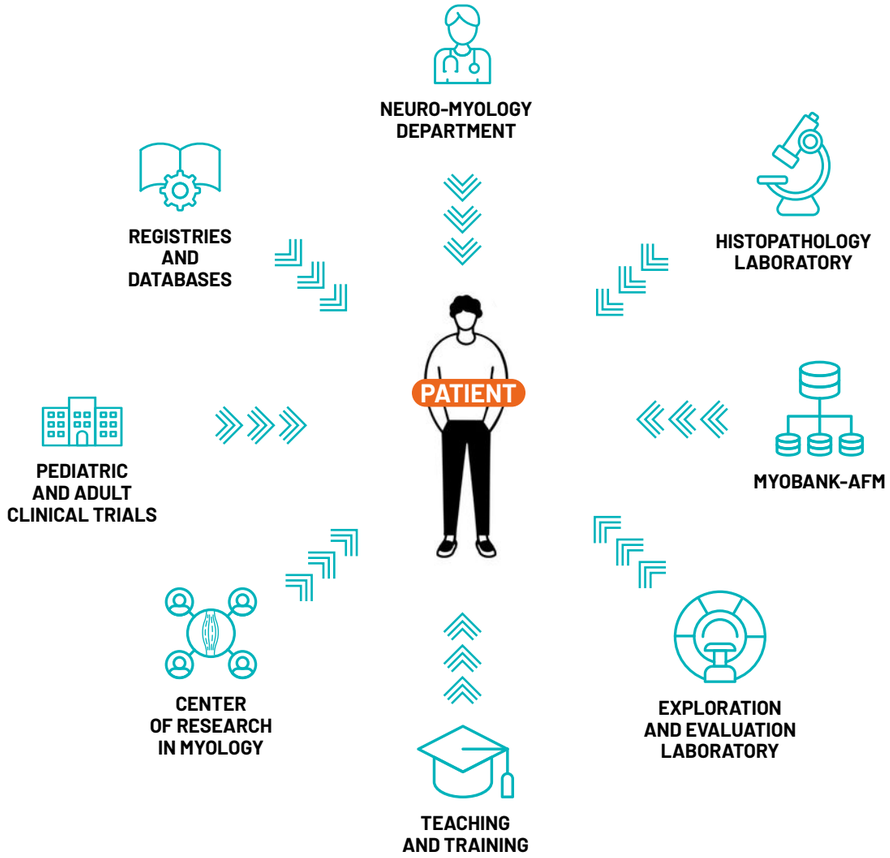


168 international scientific publications	**1** specialized adult consultation service	**1** physiotherapy and psychology center: Myocare
9 research teams	**6** technology and bioinformatics platforms	**4** experts laboratories (assessment, imaging, histopathology, biological resource center)
2 pediatric and adult clinical trial platforms: I-Motion	**1** registry and database service	**40** patent families

TOWARDS A MYOLOGY FOUNDATION

A pioneering foundation dedicated to cutting-edge medecine

Without muscles, it would be impossible to move, walk, talk, laugh, or even eat or breathe... Beyond movement and mobility, muscles interact with many organs and biological mechanisms and **are essential to our overall health.**

Faced with the major challenges and societal issues posed by sedentary lifestyles, an aging population, and the rise of non-communicable and chronic diseases (such as obesity, diabetes, cancer, heart failure, and respiratory failure), and with nearly 30 years of expertise and pioneering research in the field of muscle and neuromuscular diseases, the Institute of Myology Association and the AFM-Telethon have decided to create a **Foundation of Myology.**
This future foundation, unique in the world, will aim **to study, diagnose, evaluate, and treat muscles in all their states throughout life.**

Laurence Tiennot-Herment,
President of the Institute
of Myology Association

the first innovative treatments for particularly serious muscle diseases and the proliferation of trials make muscle a model organ and a source of multiple innovations.

It will also aim to **promote the development of myology as a cross-disciplinary field, the recognition of muscle as a tool for preventive healthcare, and to pave the way for major scientific discoveries in this particularly dynamic field of research** (new diagnostic methods, discovery of biomarkers, breakthrough therapeutic approaches). Indeed, the first innovative treatments for particularly serious muscle diseases and the proliferation of trials make **muscle a model organ and a source of multiple innovations.**

The Foundation of Myology will be a Recognized Public Interest Foundation (FRUP), founded by the Institute of Myology Association and the AFM-Telethon. It will be housed in a new 10,000 m² building at the junction between the city and the hospital, in the new Austerlitz district, the first stone of which was laid on June 17th, 2025

FONDATION DE MYOLOGIE
muscle

A new 10,000 m² building for 2028, located "between the city and the hospital", comprising a "research" center, an innovative technology center, and a "clinical evaluation" and testing center, as well as cross-disciplinary teaching, training, coordination, and support activities.

2024	**20th DECEMBER** Signing of the deed of sale for the land in the Austerlitz joint development zone, adjacent to the Pitié-Salpêtrière Hospital
2025	**17th JUNE** Laying of the foundation stone **23th JUNE** Start of construction
END OF 2027	Delivery of the building
2028	Move into the premises

HQE* certified architectural project

Sustainable building

*High Environmental Quality

Image credits: Jeudi Wang, architects Brunet Saunier & Associates, Belval & Parquet architects

2024
366 days of commitment

6TH FEBRUARY

GALA HOPE, dance for research

The Casino de Paris hosted the second edition of the Hope Gala. An exceptional evening that brought together big names in French and international dance, in aid of research and projects run by the Institute of Myology Association.

Find out more about this edition of the Hope Gala

The Institute's scientific and clinical teams collaborate on numerous **international research projects:** DREAMS, ENTRY-DM, ERDERA, GENOTHER, PRIORITY...

13TH MARCH

Call for a National Muscle Plan

The 1st Muscle Conference, organized in 2023 by the Institute of Myology and AFM-Telethon, highlighted the crucial role of muscle as a public health issue. An opinion piece published in the newspaper *Le Monde* on March 13th, 2024, highlighted the call for a National Muscle Plan signed by more than 40 prominent figures from various backgrounds in order to give myology, the science and medicine of muscle, the means to structure itself and strengthen research for the benefit of everyone's health.

Read more about this call for a "Muscle Plan"

RENDEZ-VOUS | 7

Le Monde

Un « plan muscle » s'impose l'année des JO

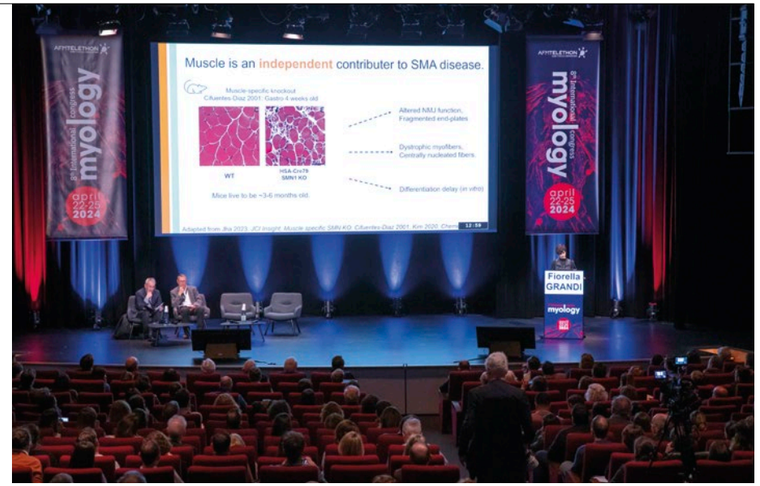
TRIBUNE - Sportifs et scientifiques s'allient pour demander que la médecine du système musculaire, essentiel à tout âge, soit désormais mieux reconnue et dispensée

APRIL

Experts from the Institute of Myology attend Myology 2024 congress in large numbers

Nearly 100 experts from the Institute of Myology participated in the AFM-Telethon International Myology Congress at the end of April 2024, which brought together more than 1,100 experts from around the world in Paris. The work of the Institute's scientific and clinical experts was highlighted through eight oral presentations and 46 posters.

Watch the video interviews with the experts



1ST - 7TH JUNE

"Muscle Week" designated "Grande Cause Nationale 2024" *

The Institute of Myology and AFM-Telethon organized the second edition of "Muscle Week". Its goal: to raise awareness among as many people as possible about the crucial importance of muscles for our health. This event, labeled "Grande Cause Nationale 2024" (Great National Cause 2024) and dedicated to physical activity and sports in a year marked by the Olympic and Paralympic Games in Paris, was a resounding success nationwide with several highlights: a stand at Roland-Garros, a workshop at the National Assembly, symposiums with sports federations (FFEPGV, FFHM), and an Open Day at the Institute of Myology, which welcomed nearly 200 visitors.

Watch the video of the Open Day at the Institute of Myology

* National Cause of the Year 2024

8TH - 12TH OCTOBER

Awards at the World Muscle Society (WMS) Congress

Three scientific experts from the Institute of Myology were honored at the 29th WMS International Congress, which took place from October 8th to 12th, 2024, in Prague, bringing together more than 1,500 international experts from 70 countries. An oral presentation and 50 posters were presented by the Institute's experts

Our Strategic Plan 2024-2027

In 2024, a new strategic plan was launched (2024-2027). Its ambition is to make the Institute the standard-bearer for myology for the benefit of patients and society. This plan consists of four strategic priorities:

- **implementing the scientific strategy**
- **promoting the fluidity and efficiency of the healthcare pathway co-constructed with the patient**
- **deploying a training program and targeted information tailored to the various stakeholders**
- **supporting the transformation of our organization**

An active caseload of
36,600
patients

5,183
neuromuscular consultations for adults

1,669
psychology consultations

160
pediatric consultations

1,538
day hospitalizations

“I-Motion allows complex trials, often international, to be conducted in a suitable environment.”

Marina Colella,
Deputy Director of I-Motion

CLINICAL ACTIVITIES AND TRIALS

Appropriate care and long-term follow-up for patients with neuromuscular diseases

The clinical care of patients with neuromuscular diseases is at the heart of the Institute of Myology’s activities. This is carried out through several departments: **the Neuro-Myology Department,** the reference center for neuromuscular diseases in the North/East/Île-de-France region and for channelopathies, **two clinical trial platforms** (I-Motion Pediatric and Adults) and **the Registries and Databases Department.**
A new department, **Myocare**, has been created, bringing together physical therapists and psychologists.

The Neuro-Myology Department,

France’s reference center for neuromuscular diseases and a member of the European Euro-NMD network, is part of the AP-HP hospital services at the Pitié-Salpêtrière Hospital. It brings together all the expertise and specialties related to neuromuscular patients to provide genetic counseling, accurate diagnosis, optimal care, access to innovative treatments, and psychological support. Several **neuro-myologists** in the department are responsible for clinical trials, natural history studies, and registries.
Within the Neuro-Myology Department, in the Myocare center, **the team of psychologists** focuses on the psychosocial impact of neuromuscular diseases on patients and their families.
Their research in the humanities and social sciences, focused on neuropsychology, enables the study of neurocognitive and socio-emotional disorders (in children and adults) and the impact of early neurogenetic vulnerabilities, particularly neuromuscular ones.

15
pediatric clinical trials conducted at I-Motion
(Trousseau Hospital)

44
adult clinical trials conducted at I-Motion
(Pitié-Salpêtrière Hospital)

I-Motion team

The I-Motion clinical trial platforms

As operational hubs for clinical research, the I-Motion Pediatric platform (created in 2015 at Trousseau Hospital) and the I-Motion Adult platform (opened in 2019 at Pitié-Salpêtrière Hospital) offer innovative treatments to patients with neuromuscular diseases in a secure environment. In partnership with leading hospital departments and supported by specialized teams, they host therapeutic trials and natural history studies that provide a better understanding of how diseases progress.

The Registries and Databases department

is dedicated to the development and monitoring of nine databases, registries, observatories, and cohort studies in the Île-de-France region and at the national level. The main objectives are to collect epidemiological and natural history data, evaluate the effects of treatments, and prepare for future therapeutic trials. It works in conjunction with the Myodata platform, which is also responsible for the biostatistics and bioinformatics aspects of research projects.

Physical activity recommended for neuro-muscular diseases

In a contribution to **Médicosport-santé**© published on the **vidal.fr** website, researchers and clinicians from the Institute of Myology shared the current state of knowledge regarding physical activity in neuromuscular diseases.
They made training recommendations for different groups of pathologies, confirming that adapted physical activity is safe and has potential positive effects on the progression of the disease and on patients' mental health.

DM1: understanding and managing cognitive disorders through fun cognitive remediation

Myotonic dystrophy type 1 (DM1) is a rare neuromuscular disease that affects the muscles, but also certain neurological functions: attention, memory, emotion regulation, and social interactions. However, these cognitive difficulties are little known and rarely addressed. At the Institute of Myology, a pilot group was launched in 2024 to address this issue, on the initiative of Sabrina Sayah and Thibaud Guillopé, clinical psychologists specializing in neuropsychology in the Psychology and Neuropsychology team of the Neuro-Myology department.

The program is based on what is known as cognitive remediation: a structured method, in small groups, which aims to help participants understand their own cognitive mechanisms and find concrete strategies to function better on a daily basis. Here, the functions targeted are social: knowing how to interact, express emotions, regulate impulsivity, and plane actions.

During twelve monthly sessions lasting an hour and a half, six patients work with a set of cards designed to address specific situations: questions, role-playing, and possible solutions. "This game is a way of training social skills. The more you do it, the better you get, just like in sports", explains Thibaud Guillopé. Participant Marie-Hélène says, "My parents noticed that I got upset less quickly, spoke more quietly, and took more time to listen to others. They saw a real improvement".

The team hopes to expand this model to other neuromuscular disorders.

The Psychology and Neuropsychology team at the Neuro-Myology Department, including Sabrina Sayah and Thibaud Guillopé.

Two reference centers at the Institute of Myology

38 reference centers in the FILNEMUS neuromuscular disease network have been accredited for five years until 2024.
The Institute's Neuro-Myology Department includes two reference centers for neuromuscular diseases in the North/East/Île-de-France region: the muscle channelopathies reference center coordinated by Prof. Bertrand Fontaine and the neuromuscular diseases reference center coordinated by Dr. Tanya Stojkovic.
Comprising a multidisciplinary team with solid clinical expertise, particularly in therapeutic trials, the neuromuscular disease reference center is a driving force at the national level and is also very involved at the European level through the Euro-NMD network, which promotes the sharing of expertise on these rare and complex diseases.

Questions for...

DR MARINA COLELLA, Deputy Director and Head of Medical Staff at I-Motion, and SAADANE KIROUANI, Deputy Director and Head of Non-Medical Staff, discuss the missions of the I-Motion platforms

What is the mission of the I-Motion platforms?

S.K. The Institute of Myology wanted to equip itself with facilities capable of testing treatments under the best conditions by creating two integrated platforms within hospitals for patients, with dedicated teams and premises: I-Motion Pediatric at Trousseau Hospital and I-Motion Adults at Pitié-Salpêtrière Hospital. These trials are aimed at patients with often serious diseases that progress at varying rates. The goal is therefore to act early, with a high level of quality and safety.

M.C. Pediatric I-Motion was created first, in 2015, to accelerate the arrival of innovative therapies for young patients and thus provide a specialized, responsive structure for conducting clinical trials with French or foreign sponsors. From the outset, the platform has relied on a collective dynamic between AP-HP, AFM-Téléthon, Sorbonne University, and the Institute of Myology. Attached to Prof. Vialle's orthopedic surgery department at Trousseau Hospital, this structure makes it possible to host complex, often international trials in a suitable pediatric environment.

What conditions do you treat and in what trials?

M.C. We monitor and treat various conditions, including Duchenne muscular dystrophy and spinal muscular atrophy (SMA). A gene therapy study in Duchenne muscular dystrophy, coordinated by the Généthon laboratory, is expected to enter its pivotal phase in the fall of 2025, and we are eager to participate.

S.K. In 2024, we participated in around 40 trials, focusing on innovative therapies such as gene therapy, but also more traditional pharmacological therapies for diseases such as Becker muscular dystrophy, FKRP-related limb-girdle muscular dystrophy, and myotonic dystrophy type 1 (MD1). We also collaborate with the hospital's neurology, intensive care, and internal medicine departments to monitor patients with inflammatory myopathies, myasthenia gravis, and neuropathies in clinical trials.

What were the highlights of 2024 and what are your prospects for the future?

M.C. In pediatrics, the year was marked in particular by the launch of the LAMA2 registry. This is a two-year national multicenter observational project that aims to better characterize the disease and prepare for future therapeutic trials. We hope that it will provide useful data for the identification of biomarkers.

S.K. On the adult side, 2024 was a year of consolidation. We conducted phase 1 to 3 trials, with a good patient recruitment rate. For 2025, we are preparing around 15 new protocols, which will allow us to maintain a relatively constant level of activity.

30+
experts (doctors, physical therapists, engineers, researchers, technicians)

6
Myotools, including 4 patented (MyoGrip©, MyoPinch©, MyoWrist, MyoQuad©, MyoAnkle, MoviPlate©)

5
centers of excellence

34,746
samples available for research, stored in the Myobank-AFM biological resource bank

Collection of
14,939
muscle biopsies

Muscle is at the heart of our research and assessments thanks to cutting-edge technologies developed in our laboratories."

Fabrice Chrétien, Director of the Neuromuscular Investigation Center

NEUROMUSCULAR EXPLORATION AND EVALUATION

Studying muscle and evaluating the effectiveness of innovative therapies for patients

The Neuromuscular Investigation Center comprises **three laboratories, a new Myocare department, and a biological resource bank** bringing together some 30 physicians, physical therapists, engineers, researchers, and technicians.
The neuromuscular physiology and evaluation laboratory, the magnetic resonance imaging and spectroscopy laboratory, the histopathology laboratory, the Myobank-AFM biological resource department and Myocare all benefit from unique expertise in muscle research and collaborate with all the Institute's teams, offering services and advice to industrial and academic partners in the context of clinical trials or collaborations.

The neuromuscular physiology and evaluation laboratory

evaluates patients with neuromuscular diseases throughout the course of their pathology, with or without treatment, in order to detect loss or recovery of neuromuscular abilities. Committed to a constant process of innovation, the laboratory has developed dedicated **tools, Myotools, and specific assessment devices and methods** to optimize the assessment and monitoring of neuromuscular patients.
Since 2023, an e-learning platform dedicated to Myotools has been deployed to train and certify more than 50 physical therapists and other medical professionals involved in dozens of clinical trials around the world.

The Myobank-AFM biological resource bank

aims to facilitate research in the field of neuromuscular diseases through its mission to collect, prepare, store, and make available fluid and tissue samples from patients affected by these types of diseases. This service helps teams comply with regulations on "research involving human subjects" (RIPH), particularly in the context of requests for clinical trials. It is accessible to all research teams, whether French or foreign. More than 1,000 samples were made available to research teams in 2024.

The histopathology laboratory

analyzes muscle biopsies from patients with neuromuscular diseases for diagnostic and research purposes. It is involved in every stage of the process, from muscle biopsy to diagnosis, including sample freezing and various muscle tissue staining techniques, including electron microscopy in certain cases. Created more than 20 years ago, this laboratory has a collection of nearly 15,000 muscle biopsy samples collected over the years, which is unique in the world.

The Nuclear Magnetic Resonance (NMR) imaging and spectroscopy laboratory

develops innovative imaging methods to monitor the progression of neuromuscular diseases, whether treated or untreated. It analyzes muscle, including during exercise, in terms of various aspects (contrasts) such as volume, fat percentage, pH, etc., which the laboratory teams quantify. The Holy Grail is to find contrasts that serve as very early markers and predictors of disease progression.

The Myocare division

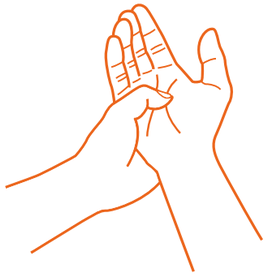
, created in 2024, brings together physiotherapy and psychology activities, covering patient care, assessments, clinical research, and patient recommendation and information projects.

Paving the way for *in vivo* microscopy of the entire muscle

ERICKY CALDAS DE ALMEIDA ARAUJO, a researcher at the NMR imaging laboratory of the Institute of Myology, has been awarded a "Young Researcher" grant (JCJC) from the French National Research Agency (ANR). This program supports ambitious research projects carried out independently by young scientists. With SPECIFICIT2Y, Ericky is developing an innovative MRI method that is both more sensitive and more specific, to refine the monitoring of neuromuscular diseases. The aim is to pave the way for *in vivo* microscopy of the entire muscle, capable of revealing alterations in the water distribution that were previously only accessible by biopsy, a localized invasive method. This approach is based on detailed biophysical modeling of MRI signals, which provide information on the distribution and circulation of water between the different histological compartments of muscle tissue. In the event of inflammation, fibrosis, or other early damage, these signals change in specific ways. Analyzing these signals would make it possible to quantify targeted pathophysiological processes and closely monitor their progression, in order to better characterize the natural history of diseases and more accurately assess the effects of treatments. The project was selected in the ANR's 2024 generic call for projects, in the "Technologies for Health" category.

CEEN - NMR Imaging and Spectroscopy Laboratory Team

Study on the link between hand muscle strength and function



In children with Duchenne muscular dystrophy (DMD) or spinal muscular atrophy (SMA), the hand can remain surprisingly functional, even when the muscles have lost a great deal of strength.
It was this observation that led the functional assessment team at the Institute of Myology to launch the EVAL study.
The goal: to better understand the gap between measured strength and what children can actually do in everyday life, in order to adapt monitoring tools. Certain movements remain possible despite greatly reduced strength. The study combines several simple methods: a hand grip test, a timed exercise involving tapping on a plate, and questionnaires on daily activities. The data will be fed into a shared database, which will be useful for monitoring changes in functional abilities in several neuromuscular diseases and for better targeting therapeutic trials.

Questions for...

CONSTANTIN SLIOUSSARENKO, researcher at the NMR imaging laboratory at the Institute of Myology, an engineer by training and an imaging researcher at the Institute of Myology, he is developing an innovative approach to analyzing respiratory muscles, particularly the diaphragm, which is responsible for inspiration, using detailed modeling and artificial intelligence.

"This project paves the way for a whole-body examination that allows all muscles to be assessed at once with a single series of images, which is more comfortable for the patient."

Why focus on respiratory muscles?

C.S. Neuromuscular diseases can affect all the muscles in the body, including those involved in breathing. The diaphragm, for example, is essential but difficult to observe: it measures a few millimeters and is in constant motion. In imaging, however, any movement generates blurring or artifacts, which makes analysis unreliable. I therefore focused my work on correcting this movement to enable quantitative assessment, particularly of fatty infiltration and inflammation in the diaphragm and intercostal muscles.

What technical solution did you develop?

C.S. We adapted the MRI sequences to record respiratory movement in real time during the examination. This allows us to obtain images at different moments during inhalation and exhalation. From this series of images, we developed a movement model that we correct using artificial intelligence algorithms. These algorithms realign the images with each other to compensate for the effects of movement. This method provides reliable measurements in an area that, until now, could not be quantitatively analyzed.

What are the benefits for patients?

C.S. Conventional imaging does not allow for accurate assessment of the condition of the respiratory muscles. With this approach, it becomes possible to monitor their evolution, detect subtle changes, and better assess the effect of treatments. This represents a significant advance in documenting the natural history of certain neuromuscular diseases and improving patient follow-up. To my knowledge, this type of quantitative assessment has never been performed on these muscles before.

Questions for...

BRUNO CADOT, Director of Research at the Institute of Myology

Bruno Cadot has developed a unique tool: **a Muscle Atlas** based on thousands of images of human muscle sections. This project combines imaging, medical memory, and artificial intelligence to better diagnose and understand neuromuscular diseases.

Why did you create a muscle atlas?

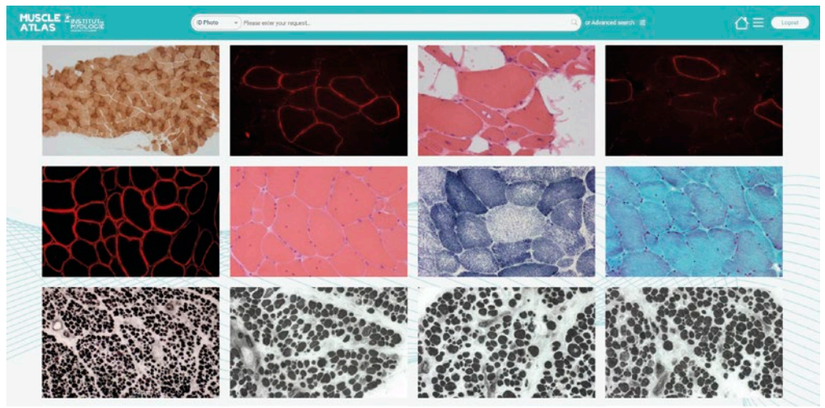
B.C. When I arrived at the Institute of Myology in 2007, I began studying the movement of nuclei in muscle fibers using imaging techniques.
This led me to take an interest in muscle sections, and then to contact the histopathology laboratory, which had a considerable volume of samples. The idea of combining our expertise to create a visual atlas, similar to those already developed in oncology, quickly took hold. Such a tool is invaluable for research, training, and diagnostic support.

What is the basis for this project?

B.C. The Institute's histopathology laboratory has analyzed samples from more than **20,000 patients** since the 1990s. This collection is an exceptional resource, consisting of slides and digital images. To date, **approximately 6,500 sections have been digitized,** with the goal of reaching **20,000 referenced images** within three years. This corpus will form a shared repository, intended to inform professionals involved in the management of muscle diseases.

What concrete benefits can be expected?

B.C. The diagnosis of neuromuscular diseases often relies on detailed analysis of the muscle under a microscope. However, this process can be extremely time-consuming: some patients wait five to six years before receiving a complete diagnosis. By bringing together these thousands of images in one place, it becomes possible to design a diagnostic aid tool based on artificial intelligence. Already tested on several congenital diseases, this system has **a success rate of over 90%.** Such an advance would significantly reduce diagnostic uncertainty. To our knowledge, no equivalent project has been developed elsewhere.

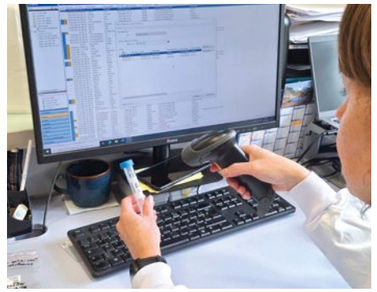


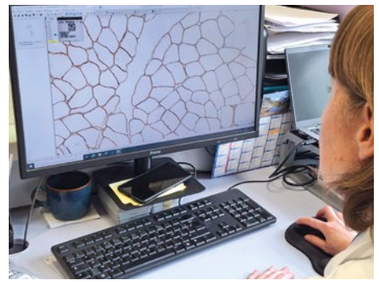
Muscle Atlas:
a database of more than 6,500 muscle biopsy images available for research

Slide scanner for digitizing muscle biopsies

Digital transformation for more accurate diagnosis

The histopathology laboratory is involved in everything from muscle biopsy to diagnosis, including sample freezing and various muscle tissue staining techniques, including electron microscopy in certain cases.
It analyzes muscle biopsies from patients with neuromuscular diseases for diagnostic and research purposes.
Currently undergoing digital transformation since 2024, this laboratory, renowned for its morphological expertise, has benefited from significant investments in equipment, professional training, cloud computing deployment, and the upcoming integration of artificial intelligence. These developments will enable it to improve diagnostic accuracy, accelerate research, and enhance training, while consolidating its position as a center of excellence in the field of neuromuscular diseases. This evolution will involve the analysis of high-definition images, the automation of certain tasks, the improvement of the traceability of samples and associated data, and finally the analysis of large amounts of data, which will accelerate the understanding and treatment of rare diseases.





150
researchers, engineers, technicians

9
multidisciplinary research teams

168
international scientific publications

“We explore areas that were previously inaccessible in order to better understand how cells interact with each other and what goes wrong in the event of disease.”

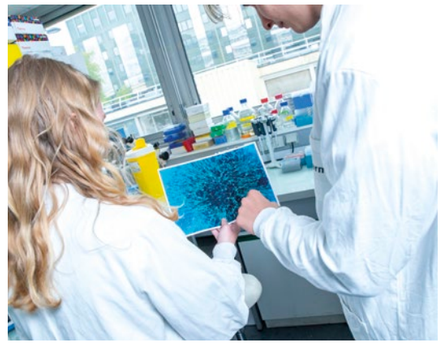
Stéphane Vassilopoulos,
Researcher at the Center of Research in Myology

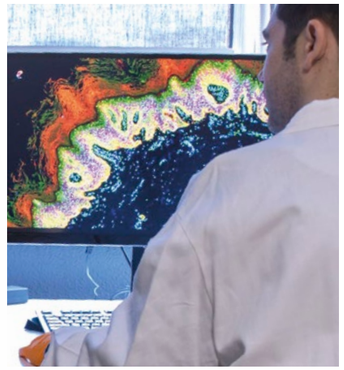
RESEARCH IN MYOLOGY

Understanding muscle and discovering new therapies for patients

The research teams at the Institute of Myology conduct **translational research,** bridging the gap between **basic and applied research.** Their goal is to rapidly transform scientific discoveries into tools, treatments, or diagnostics for clinical practice (clinical studies and trials). In association with the Institute’s other divisions (Neuro-Myology Department, Registries and Databases, I-Motion Pediatric and Adult Clinical Trials Division, Neuromuscular Exploration and Evaluation Center), the Institute’s **Center of Research in Myology** constitutes an ecosystem that promotes interaction and the emergence of innovative projects.

The Center of Research in Myology is a hub of nine multidisciplinary research teams bringing together around **150 researchers, doctors, students, engineers, and technicians** from various public and private partners (Inserm, Sorbonne University, CNRS, AP-HP, and the Institute of Myology Association). They study muscles and muscle pathologies to better understand muscles in their environment and translate these discoveries into new approaches and innovative concepts to improve care for patients with neuromuscular diseases.





Our expertise platforms

The Institute also has six shared expertise platforms that bring together state-of-the-art equipment for specific technologies. These platforms are mainly available to the Institute’s teams, but some also collaborate with external academic or private teams.

- **MyoImage:** has state-of-the-art imaging and image analysis equipment offering maximum imaging possibilities while meeting quality requirements through various approaches to conventional and electron microscopy.
- **MyoLine:** provides immortalized lines of myoblasts and fibroblasts converted into muscle cells.
- **MyoVector:** produces gene therapy vectors derived from adeno-associated viruses (AAV).
- **MyoMolBio:** monitors technological developments in order to provide innovative molecular biology tools.
- **MyoData:** offers researchers and clinicians at the Institute expertise in the organization, processing, and analysis of data from research and clinical research.
- **Avatar:** models human muscles in miniature form on microfluidic chips.

European grant to explore the effects of DM1 on the brain

GABRIELE ORDAZZO, a postdoctoral researcher at the Institute of Myology, has been awarded a **Marie Skłodowska-Curie (MSCA) grant,** one of the most prestigious in Europe. Selected from thousands of applicants, he will receive two years of funding to carry out an innovative project on the cerebral form of myotonic dystrophy type 1 (MD1). In this disease, defective RNA fragments disrupt communication between neurons and glial cells, which support and assist neurons. Using a mouse model developed in the laboratory, Gabriele will work to better understand these mechanisms, which are still largely unexplored.

ERDERA, a European alliance to better understand and treat rares diseases

Launched in September 2024, the European ERDERA alliance, coordinated by Inserm and supported by the European Union, aims to structure research on rare diseases at the continental level. This program builds on the efforts of projects such as Solve-RD by facilitating data sharing, harmonization of tools, and pooling of resources.
For Gisèle Bonne, team leader at the Center of Research in Myology, this new framework should enable us to capitalize on what already exists. Her team will participate in several aspects of the program: analyzing genetic and clinical data to better understand certain undiagnosed conditions, contributing to Treatabolome, a database of existing treatments enriched by artificial intelligence, and leading the National Mirror Group, which links French and European priorities. *"The challenge is to structure a collective, coherent, and useful response for patients"*, she concludes.

A muscle grown in the laboratory: soon to be a reality?

Every year, millions of people lose a significant amount of muscle mass as a result of an accident, illness, or surgery, leading to serious consequences: loss of mobility, chronic pain, and reduced independence. Unlike skin or bone, muscle cannot regenerate spontaneously when severely damaged.
Today, there is no solution for replacing it over a large area.
To break this deadlock, **BRUNO CADOT, Director of Research at the Institute of Myology, is coordinating Orgomus: a project that aims to create a transplantable muscle** prepared in the laboratory. This muscle is formed from human cells injected into a biomimetic collagen matrix (i.e., inspired by the natural structure of muscle). *"What is fascinating about muscle is that its function depends directly on its architecture. You cannot make a muscle contract if its cells are not perfectly aligned"*. In mice, the grafts show certain functional characteristics within a few weeks. The main obstacle now is to ensure good blood supply so that they can survive after transplantation. *"Even if we obtain a perfect muscle in the laboratory, if it is not supplied by an efficient vascular network, it will not survive"*. The first clinical trials are planned for 2028.

Questions for...

STÉPHANE VASSILOPOULOS, co-leads the "Muscle Cell Organization and Therapy of Dominant Centronuclear Myopathy" team at the Institute's Center of Research in Myology.

Observing how a neuron or muscle cell works means delving into the infinitely small. Until recently, even the most experienced scientists were unable to access certain mechanisms. That was until a unique technology was developed at the Institute of Myology based on a researcher's intuition. Stéphane Vassilopoulos shed light on a key phenomenon in the functioning of neurons in an area that was thought to be inactive. These findings were published in the prestigious journal Science (August 2024) and made the front page of Nature Structural & Molecular Biology the same year.

How was this unprecedented discovery made possible?

S.V. It is based on a combined microscopy technology that we developed at the Institute of Myology. By combining fluorescence imaging and electron microscopy, we obtain both an overview and a very precise location of certain proteins. We applied this approach to cell cultures and then added an essential step: ultrasonic stripping, which allows us to visualize the proteins of interest without losing their three-dimensionality.
Each technique enhances the other and creates unique added value with a level of detail never before achieved. It's a bit like a GPS for the infinitely small!

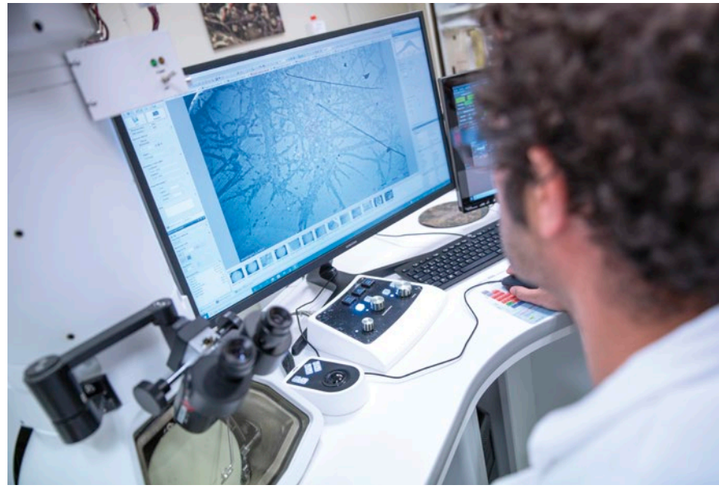
What exactly did you observe in the axon, the part of the neuron that conducts nerve signals?

S.V. Thanks to this combination of microscopes, we discovered that, contrary to what was previously believed, the very beginning of the axon contains small pits covered with clathrin, a protein that helps form small bubbles called vesicles.
These pits slip into the openings of a fine mesh located just below the neuron's membrane, a bit like a spider's web: this is the spectrin network, which supports the membrane. When this area is stimulated by a signal, the membrane folds back, the pit closes, and the vesicle enters the cell: this is endocytosis. This is the first time such a mechanism has been observed in this part of the axon.

What prospects does this discovery open up?

S.V. This technology allows us to explore previously inaccessible areas at the very heart of the muscle and associated neural structures. It helps us to better understand how cells interact with each other and what goes wrong in the event of disease. By understanding what happens in normal situations, it is possible to better identify abnormalities, such as those observed in certain myopathies where the clathrin structure is altered.

"This opens up new avenues for understanding neuromuscular disorders and exploring novel therapeutic possibilities."



Questions for...

NADINE DRAGIN, researcher at the Institute of Myology

Certain cells can be altered by endocrine disruptors, which have been observed to be associated with worsening myasthenia gravis. To better understand this phenomenon, Nadine Dragin's team is developing a mouse model in which the effects of endocrine disruptors in thymic epithelial cells are modulated.

Can endocrine disruptors promote the emergence of autoimmune diseases?

N.D. Endocrine disruptors are all around us: in plastics, cosmetics, certain foods, and even textiles. We know that they can interfere with our hormones, but one question remains: could they also disrupt the immune system to the point of contributing to the onset of autoimmune diseases? This is precisely what I am exploring in my research at the Institute of Myology. I am working more specifically on myasthenia gravis, a rare disease that causes muscle weakness, which can sometimes be very debilitating. Through this project, my goal is to better understand the role that environmental factors – including endocrine disruptors – can play in triggering autoimmune diseases.

"Our goal is to better understand the role of environmental factors, such as endocrine disruptors, in triggering autoimmune diseases, such as myasthenia gravis."

What is the origin and context of this project?

N.D. Analysis of the organs of patients with myasthenia gravis showed signs suggesting that certain substances in the environment could play a role in the disease. Traces consistent with the effect of endocrine disruptors were observed in the thymus (an organ involved in the functioning of the immune system) and in muscle. These results led the team to test these substances in the laboratory to see if they could trigger abnormal reactions. Initial tests seem to point in this direction. In 2024, a doctoral student, funded in part by the Île-de-France Region, joined the project to better understand these mechanisms.

How can these disruptors affect the immune system?

N.D. The objective is primarily preventive. Prolonged exposure to these molecules, particularly during sensitive periods such as puberty, could affect the thymus and trigger a cascade of immune events that promote the onset of disease. Once the disease has taken hold, this type of exposure seems to maintain or even aggravate existing imbalances. The project is being conducted in collaboration with the MyoData team at the Institute of Myology, which is helping us to decipher a set of biological data necessary for the study.

Questions for...

MARIO GOMES-PEREIRA, Director of Research at the Institute of Myology

In 2024, the Center of Research in Myology obtained funding from ENTRY-DM (European Network for Training and Research in Myotonic Dystrophy), a European doctoral network supported by the Marie Skłodowska-Curie Actions. This structuring project will support 14 doctoral students over four years in six countries. At the head of the consortium is Mario Gomes-Pereira, Director of Research at the Institute of Myology. Franco-Portuguese, he embodies a generation of European scientists trained at the crossroads of disciplines and cultures.

Why does this project represent an important step forward for research into DM1, the most common neuromuscular disease in adults?

M.G.-P. ENTRY-DM is not just a research project. It is a structuring program, designed to organize coherent and ambitious training within a resolutely European framework. DM1 is a complex disease that requires an integrated approach combining genetics, cell biology, neurology, and neuroscience. We are at a turning point. The first clinical trials are underway. But the disease is still poorly understood, its mechanisms are complex, and the tools for monitoring the effectiveness of treatments remain limited. It is essential to train a new generation of researchers with a broader vision.

How is the ENTRY-DM program structured?

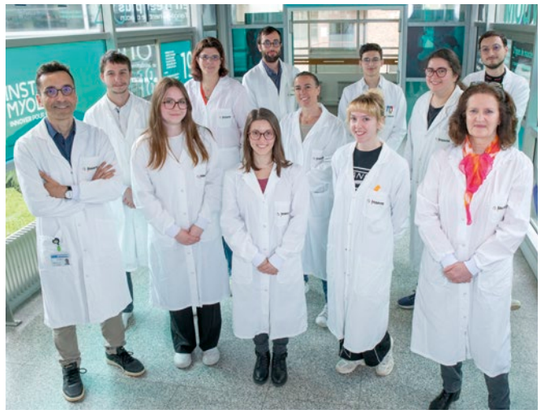
M.G.-P. The theses will be divided into three areas: development of cellular and tissue models (mimicking what happens in the disease), exploration of therapeutic strategies, and identification of biomarkers. These areas are not compartmentalized, quite the contrary. The models produced by some will be used to test molecules developed elsewhere, and the responses will be evaluated with tools generated by others. This complementarity is at the heart of the network. ENTRY-DM is an essential step in creating a strong and interconnected European research community on DM1. The scientific, administrative, and logistical coordination of the program is provided by the Institute of Myology Association in coordination with Inserm, with the support of a project manager.

What support is available for doctoral students?

M.G.-P. Doctoral students will benefit from a comprehensive training program: seminars, practical workshops, cross-disciplinary modules (ethics, bioinformatics, clinical research, etc.), a popular science blog, and two three-month placements, one of which will be in a non-academic setting. We need to train researchers who are capable of working in a wide variety of environments: public laboratories, start-ups, biotech companies, and regulatory agencies.

This project resonates with your own career path. What does the opportunity to be part of such a network mean to you?

M.G.-P. I benefited from a Marie Curie grant at the beginning of my career. I know how much it can shape a career path. ENTRY-DM is an opportunity to pass on this opportunity and support a generation of young researchers in a human, scientific, and European adventure that I hope will have as lasting an impact on their careers as it did on mine.



Mario Gomes-Pereira's research team, led by Geneviève Gourdon, at the Center for Research in Myology

AVATAR

The Institute of Myology has just launched the first work on AVATAR, an innovative platform led by researcher Andrew Ho.
The goal is to model a miniature human muscle, grown on a chip from patient cells, to better understand how it works and test treatments more accurately.

Unlike traditional models, AVATAR allows several cell types (neurons, immune cells, etc.) to be integrated into a miniaturized circulation system, recreating an environment close to that of the human body.

This technology promises to accelerate research, limit the use of animal models, and contribute to personalized medicine.

Questions for...

MAXIME JACOUPY, Director of Data at the Institute of Myology, oversees the MyoData platform

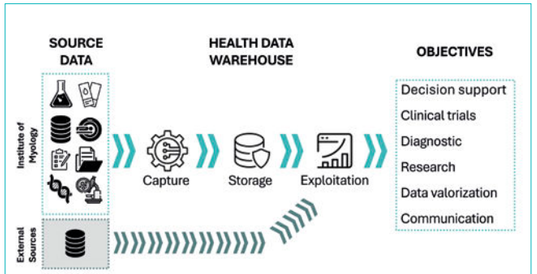
With a PhD in neuroscience and a master's degree in artificial intelligence, he embodies dual expertise at the interface of biomedical sciences and digital technologies.
His mission: to structure, ensure the reliability of, and make usable the data produced at all levels within the Institute.

Your background combines biology and data science. How is this dual training essential?

M.J. I am a researcher with additional training in data science/artificial intelligence. The platform's objective is to design tools that are useful, reliable, and understandable to researchers. This requires mastery of several languages: those of biology, algorithms, and mathematics. The analysis methodology can be learned, but a solid scientific foundation remains essential.

How is MyoData's work structured at the Institute level?

M.J. The team's activity is based on two components. The first is analytical: researchers, doctors, and engineers are supported in the processing of their data, whether it be statistics, bioinformatics, or algorithm development. The second is structural: a global database, the MyoData Data Warehouse (EDS), is currently being developed to centralize the Institute's data from clinical research, laboratories, and healthcare. It constitutes a cross-functional, secure foundation that complies with Health Data Hosting (HDS) certification requirements, which will enable new research to be carried out on this data.



The Health Data Warehouse (EDS): a centralized solution for managing muscle data from our laboratories' research and clinical activities

You are also involved in GenoTher, a biocluster dedicated to gene therapies. What is your role?

M.J. Through the MyoData EDS, we will be involved in the pre- and post-trial data analysis phase.
The aim is to develop tools to evaluate the effectiveness of treatments, anticipate patient response, and limit the use of "control arms". This work is based on approaches combining artificial intelligence and statistical modeling.

What future do you see for these practices?

M.J. Data analysis is already very advanced in oncology. In rare diseases, there is still some way to go, but that is precisely what makes the work so exciting.
Once the data is well structured and accessible, these approaches can profoundly transform the way we generate knowledge and support patients.

In 2024, MyoData supported
10 teams, centers, and platforms
at the Institute of Myology
for nearly **40** projects.

26 years
of the Myology Summer School

Over than 1,000
doctors trained

More than 80
nationalities from
5
continents

> **"I really appreciated the quality of the topics, the speakers, and the discussions. I will be able to use this new knowledge in my clinical practice."**
>
> Leighann, physician at the University of Oxford, United Kingdom, participant in the Myology Summer School

TEACHING

Promoting the dissemination of knowledge about muscles and muscle diseases and raising awareness of myology

The Institute of Myology plays an important role in providing information, training, and education to all audiences interested in myology. The aim is to promote the dissemination of knowledge about muscles and muscle diseases and thus contribute to raising the profile of myology in the medical and scientific fields. **Degree courses are offered each year at the Institute of Myology** during the academic year: **Myology Summer School (AcadeMyo), "Complexity of Life" doctoral school,** co-accredited by Sorbonne University and the École Normale Supérieure Ulm, **DIU in Myology,** in partnership with Santé Sorbonne University and Aix-Marseille II University, **Inter-University Diploma in Psychopathology and Neurological Disorders,** in partnership with Paris 8 University and Sorbonne University.

FOCUS ON...

Summer School of Myology

It is primarily aimed at healthcare professionals and scientists from abroad. Over the course of a week, participants follow an intensive program covering all neuromuscular disorders in children and adults. This theoretical and practical training is delivered in English by internationally recognized experts. It is also an opportunity to establish collaborations with professionals and teams of doctors and researchers from around the world, particularly from countries where this new medical-scientific discipline is emerging. In 2024, the summer school celebrated its 26th anniversary and the fourth edition of the new hybrid version, renamed AcadeMYO in 2020, which includes an online course platform. Twenty-four students from 18 countries came to perfect their knowledge of fundamental and clinical myology for the 2024 session. In total, more than 1,000 doctors of over 80 different nationalities from five continents have been trained since its creation in 1998.

FOCUS ON...

The DIU in Myology

Created in 1996 in partnership with Sorbonne University and AFM-Téléthon, its aim is to provide students with a coherent, structured, and modern view of neuromuscular pathology in terms of its anatomical and physiological foundations, clinical practice, complementary investigations, and developments in research. This program is open to French and foreign doctors and medical students who have completed their second cycle of medical studies.
Twenty-eight students enrolled in this DIU program for the 2024 session, which is a joint program between Sorbonne University and Aix-Marseille-II University.

FINANCIAL REPORT

Since its creation in 2005, the Institute of Myology Association has been committed to sharing financial information with its stakeholders in a transparent manner, accounting for the use of the funds allocated to it. It publishes its full annual accounts and the auditor's report in the Official Journal of Associations.

ASSETS

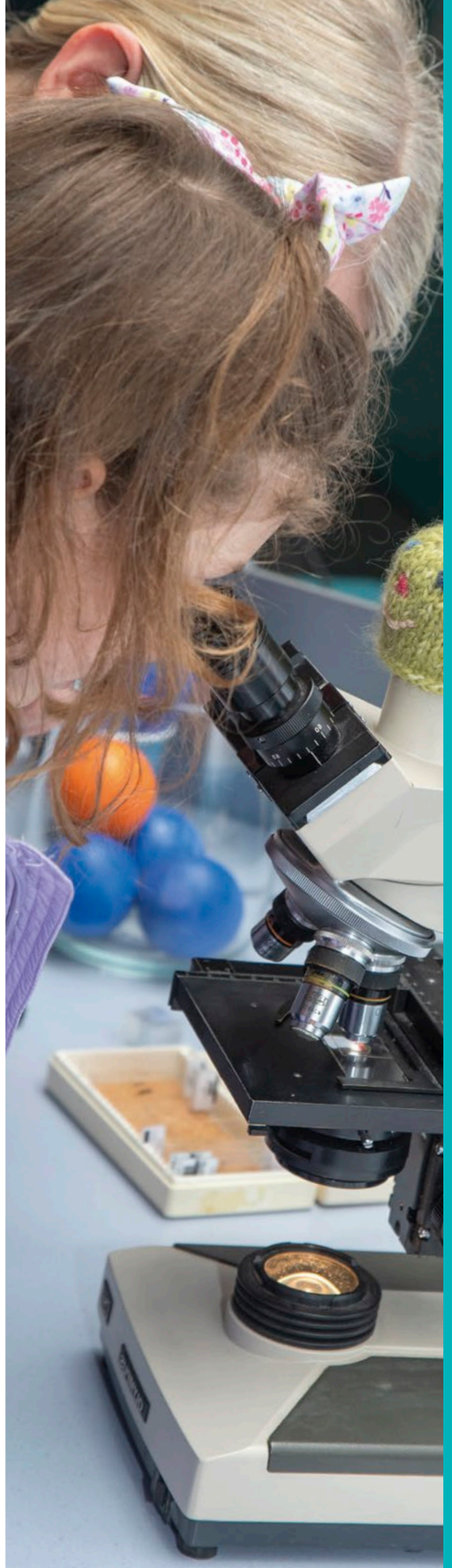
ITEMS	31/12/2024			31/12/2023
	Gross amount	Impairment	Net amount	Net amount
INTANGIBLE ASSETS	**294,977**	**289,517**	**5,460**	**11,420**
Start-up costs				
Research and development costs				
Temporary donations of usufruct				
Concessions, patents, licences, trademarks, processes, software, rights and similar assets	294,977	289,517	5,460	11,420
Other intangible assets				
Intangible assets in progress				
Advances and deposits				
TANGIBLE ASSETS	**6,944,147**	**4,495,183**	**2,448,964**	**1,811,655**
Land				
Buildings				
Technical installations, machinery and industrial equipment	3,950,799	2,082,218	1,868,581	1,317,324
Other tangible fixed assets	2,826,778	2,412,965	413,813	494,331
Restricted tangible assets				
Fixed assets in progress	74,070	-	74,070	-
Advances and deposits	-	-	-	-
Assets received by bequest or donation intended for disposal	92,500	-	92,500	-
FINANCIAL ASSETS	**10,010**	**-**	**10,010**	**10,010**
Equity investments	10,010	-	10,010	10,010
Receivables related to equity investments				
Other long-term securities				
Loans				
Other financial assets	-		-	-
FIXED ASSETS	**7,249,134**	**4,784,700**	**2,464,434**	**1,833,085**
INVENTORIES AND WORK IN PROGRESS	**-**	**-**	**-**	**17,010**
Raw materials, supplies				
Work in progress goods and services				
Intermediate and finished products				
Merchandise				
Advances and deposits paid on orders	-	-	-	17,010
RECEIVABLES	**12,099,873**	**248,906**	**11,850,967**	**11,885,951**
Trade receivables, user receivables and related accounts	4,444,217	248,906	4,195,311	6,549,016
Receivables received to bequests or donations	122,336	-	122,336	-
Other receivables	7,533,320	-	7,533,320	5,336,935
MISCELLANEOUS	**13,065,174**	**-**	**13,065,174**	**14,595,881**
Marketable securities				
Cash instruments				
Cash	13,065,174	-	13,065,174	14,595,881
ACCOUNTS RECEIVABLE	**510,809**	**-**	**510,809**	**274,423**
Prepaid expenses	510,809	-	510,809	274,423
CURRENT ASSETS	**25,675,856**	**248,906**	**25,426,950**	**26,773,265**
TOTAL ASSETS	**32,924,990**	**5,033,606**	**27,891,384**	**28,606,350**

LIABILITIES AND EQUITY

ITEMS	31/12/2024	31/12/2023
EQUITY	**14,842,422**	**13,600,833**
Equity without right of recovery		
Statutory equity		
Additional equity		
Association equity without right if recovery (bequests, donations, government subsidies, renewable assets)		
Statutory equity	2,354,563	2,354,563
Additional equity		
Contributions		
Bequests and donations		
Results under control of third party funders		
Statutory or contractual reserves		
Reserves for entity projects	8,222,269	8,203,799
Including reserves for social and medical-social activities under controlled management		
Reserves	70,731	178,792
Other reserves		
Retained earnings	2,667,957	1,987,257
Including retained earnings for social and medical-social activities under controlled management		
Surplus or deficit for the financial year	1,261,970	591,109
Consumable equity		
Investment subsidies	264,932	285,313
Regulated provisions		
Owners' rights (commodat)		
DEDICATED FUNDS	**1,613,797**	**1,504,533**
Funds carried forward related to bequests or donations	189,731	
Dedicated funds	1,424,066	1,504,533
PROVISIONS FOR RISKS AND CHARGES	**1,092,448**	**855,555**
Provisions for risks and charges	1,092,448	855,555
DEBTS	**10,342,717**	**12,645,429**
Loans and debts with credit institutions	1,433,396	2,035,687
Miscellaneous loans and financial debts		
Advances and deposits received	18,556	18,556
Trade payables and related accounts	1,377,938	1,025,163
Debts related to bequests or donations	25,105	-
Tax and social security debts	2,598,494	2,512,508
Debts on fixed assets and related accounts	-	-
Other debts	2,102,093	4,115,381
Cash instruments		
Deferred income	2,787,135	2,938,134
TOTAL LIABILITIES	**27,891,384**	**28,606,350**
Net income for the year (in cents)	*1,261,970.58*	*591,109.18*
Total balance sheet (in cents)	*27,891,384.17*	*26,606,350.38*

INCOME STATEMENT

ITEMS	31/12/2024	31/12/2023
Contributions		
Sales of goods and services		
Sales of goods		
Sales of services *Of which sponsorships, sales of services relating to social and medico-social activities*	2,768,590	3,741,491
Revenue from third-party funders		
Public tenders and operating subsidies	252,642	429,226
Financial contributions from pricing authorities relating to social and medico-social activities		
Payments from founders or consumption of consumable endowments		
Resources linked to public generosity		
Manual donations	490,501	530,428
Sponsorships		
Bequests, donations and life insurance	43,502	-
Financial contributions	13,159,993	10,247,916
Reversals of amortization, provisions and transfer of charges	965,465	1,267,191
Use of dedicated funds	590,048	518,624
Other income	192,477	463,456
TOTAL OPERATING INCOME	**18,463,218**	**17,198,332**
Other purchases and external expenses	3,577,826	3,922,341
Financial assistance	2,433,350	2,290,026
Taxes, duties and similar payments	679,593	674,446
Salaries and wages	6,167,041	5,830,962
Social security contributions	2,873,008	2,705,446
Provisions for depreciation and impairment	387,553	431,887
Provisions for impairment of current assets	235,136	13,770
Provisions	303,897	160,361
Subsidies paid by the association		
Transfers to dedicated funds	509,581	389,020
Other expenses	79,359	216,655
TOTAL OPERATING EXPENSES	**17,246,344**	**16,634,914**
OPERATING INCOME	**1,216,874**	**563,418**
Financial income from investments		
Financial income from other marketable securities and fixed asset receivables	747	
Other interest and similar income	4,179	5,537
Reversals of provisions and transfer of expenses		
Positive exchange differences	11	465
Net income from disposals of marketable securities		
TOTAL FINANCIAL INCOME	**4,937**	**6,002**
Financial allocations to depreciation and provisions		
Interest and similar expenses	48,428	51,430
Negative exchange differences	2,625	1,405
Net expenses on disposal of marketable securities		
TOTAL FINANCIAL EXPENSES	**51,053**	**52,835**
FINANCIAL RESULT	**(46,116)**	**(46,833)**
Exceptional income from management operations	80,656	11,402
Exceptional income from capital transactions	28,681	11,614
Reversals of provisions, impairments and transfers of expenses		
TOTAL EXCEPTIONAL INCOME	**109,337**	**23,016**
Extraordinary expenses on management transactions	157,122	13,714
Extraordinary expenses on capital transactions		
Provisions for depreciation, impairment and provisions	2,106	28,123
TOTAL EXTRAORDINARY EXPENSES	**159,228**	**41,837**
EXTRAORDINARY INCOME	**(49,891)**	**(18,821)**
Tax Research Credit	(141,103)	(93,345)
TOTAL INCOME	**18,577,492**	**17,227,350**
TOTAL EXPENSES	**17,456,625**	**16,729,586**
RESULT FOR THE YEAR	**1,261,970**	**591,109**



DONATIONS IN SHARES

€491,000 CRAISED IN 2024 THANKS TO THE GENEROSITY OF OUR DONORS TO FUND OUR PROJECTS (RESEARCH AND FOUNDATION)

€117,840
Strategic coordination of the Myology Foundation project (project management, architectural, legal, and tax studies, etc.)

€63,830
Funds reserved for project development and the construction of the future Foundation

€309,330
Awareness-raising, communication, and fundraising for the development of the Foundation project

NATIONAL CAUSE & MUSCLE A PUBLIC HEALTH ISSUE

- **6th February 2024:** Second edition of the Hope dance gala to support research and our Myology Foundation project.
- **1st – 7th June 2024:** Second edition of "Muscle Week", certified GCN*2024.www.lemuscle.fr
- **Summer 2024**: Awareness-raising activities and meetings at the Club de France during the Olympic and Paralympic Games.

STRONG CAMPAIGNS
INFORMATION, AWARENESS AND CALLS FOR DONATIONS

- **2024:** Monthly "Muscler la Vie" newsletter campaigns and "Online Donors" email campaigns (€150,000 raised).
- **April 2024**: Spring mailing, focus on IFI** (6,600 mailings, €48,000 raised).
- **December 2024:** End-of-year mailing, focus on IR and IS*** (50,000 mailings, €40,000 raised).

*GCN: Grande Cause Nationale (Major National Cause), ** IFI: Impôt sur la Fortune Immobilière (Real Estate Wealth Tax), *** IR: Impôt sur les Revenus (Income Tax), IS: Impôt sur les Sociétés (Corporate Tax)

DONATIONS ALLOCATED TO SCIENTIFIC PROJECTS

(MyoImage imaging platform, research projects on Myotonic Dystrophy Type 1 - MD1, etc.) :
€63,000 in 2024 (the unused portion of these donations has been placed in dedicated funds).

Some actions open up new perspectives. We are going to build a pioneering foundation for muscle research, bringing together the best expertise.

EVERY GESTURE IS A DONATION: SUPPORT OUR MYOLOGY FOUNDATION PROJECT

MAKE A DONATION
online, by bank transfer, or by check

TAX DEDUCTION FOR MY DONATION
Take advantage of tax benefits to support your gesture with a tax reduction of 60%, 66%, or up to 75% depending on your situation!

CONTACT
Donor Services Ambition Myology Foundation
07 80 91 40 21 - donateurs@ambitionfondationdemyologie.org

ambitionfondationdemyologie.org

INSTITUTE OF MYOLOGY
CURE THROUGH INNOVATION

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