AFMTÉLÉTHON
CURE THROUGH INNOVATION

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JULY 2024

The values

OF AFM-TELETHON ARE THOSE OF PATIENTS AND THEIR PARENTS WHO ARE DETERMINED TO DO EVERYTHING IN THEIR POWER TO DEFEAT THE DISEASE

MAKING CHOICES **DETERMINATION AND CONVICTION**

Since it was created, AFM-Telethon does its utmost to fulfil its ambition and conviction that a cure is possible.

BEING HELD ACCOUNTABLE **TRANSPARENCY**

More than a legal requirement, transparency is an ethic. Ever since the first Telethon, our organisation has committed to reporting faithfully on the use of donations.

RISING UP AGAINST NEGLECT AND IGNORANCE **REVOLT**

Revolt is a founding value of AFM-Telethon, the revolt of parents who refuse to give in to fate and resignation.

SUCCEEDING **RIGOUR AND EFFICIENCY**

This is the golden rule AFM-Telethon established in order to achieve its goals, because it operates in complex areas with limited resources.

A patients' organisation,

patients and relatives fighting neuromuscular diseases, rare genetic disorders that kill muscle after muscle.

An organisation bringing together volunteers and employees,

guided by patients' interest and the urgency of evolving conditions.

A goal which remains unchanged: **to conquer the disease.**

A strategy of general interest

which gives priority to boldness and innovation to the benefit of those affected by a rare diseases and more generally by a disability.

Exceptional popular support through the Telethon its annual fundraising event.

THREE MISSIONS at the heart of *our action*

CURE

Curing neuromuscular diseases, which are rare, progressive and severely disabling, is the Association's priority mission. To achieve its objective, the AFM-Téléthon has chosen to pursue a general interest strategy that benefits all rare diseases, and even beyond. The Association creates and develops laboratories and tools to advance our understanding of diseases, the development of innovative therapies based on our knowledge of genes and cells, and their application to humans. Its guiding principles: innovation and therapeutic effectiveness.

CARE

Support patients and their families to live their lives according to their goals by reducing the impact of the disease in everyday life. That's why AFM-Telethon stands for the rights of patients in a wide range of fields from access to diagnosis and relevant healthcare, to local support, ever seeking innovative solutions that meet patients' needs.

COMMUNICATE

Ever since it was created, AFM-Telethon used communication as an essential tool to raise awareness on rare diseases, disseminate and explain research findings, promote therapeutical progress, and advocate for patients to bring their fight to the forefront.

AFM-TELETHON'S *galaxy*

BIOTHERAPIES INSTITUTE FOR RARE DISEASES

INSTITUT DE MYOLOGIE
European reference centre for muscles (care – research – training) and its clinical trial platforms for children and adults (I-Motion Institutes)

I-STEM
First French stem cell research centre for genetic diseases

GENETHON
Centre of expertise in the design, development and production of gene therapy drugs for rare diseases

ATAMYO THERAPEUTICS
A mission-led biotechnology company, created by Genethon, focused on the development of gene therapies for Limb-Girdle Muscular Dystrophies

SEED FUND
"Innovative biotherapies for rare diseases" seed fund, created in partnership with Bpifrance, the French public investment bank

H-MRB

YPOSKESI
An industrial facility dedicated to the production of biotherapies

GENOSAFE
Service provider
Control and safety of biotherapeutic products

AMPLEIA
Preclinical and clinical development of drug candidates in rare diseases

GENOPOLE® EVRY
Research campus, centre of excellence, in genome and post-genomic studies

A PATIENTS AND FAMILIES ORGANIZATION

AFMTELETHON

A board of trustees
consisting of volunteer patients and relatives of patients

Volunteer advisers
- The finance committee
- The scientific council

Operational departments
- Scientific
- Medical action
- Actions for families
- General secretariat
- Marketing and development of resources
- Public affairs
- Communication
- Fundraising and mobilisation
- Human resources

Génocentre
A conference centre to contribute to the influence of Genopole® Évry

Our support networks for families
16 teams of professionals in different regions of France (regional departments)

72 delegations in the French departements made up of volunteers affected by disease

8 interest groups, volunteers who are experts in their specific diseases

Places to stay and respite care facilities
The Yolaine de Kepper centre (A residential care home for highly-dependent patients; Gâte Argent: an innovative concept of accommodation and services; Respite home: "Le Village Répit Familles®" La Salamandre), the houses of Étiolles and La Hamonais, an apartment in Paris

Our Telethon network
147 local teams of volunteers which coordinate the development of fundraising events during the Telethon in each French department and abroad

THE RARE DISEASES PLATFORM
A unique resource centre for rare diseases

Rare diseases info service
French Foundation for Rare Diseases
Rare diseases alliance
Orphanet
Eurordis

VILLAGE REPIT FAMILLES
Les Cizes respite home in Jura, France

AFM PRODUCTIONS
Movies to spread knowledge and public awareness

○ Entity created and predominantly controlled by AFM-Telethon

◌ Entity created and/or financed by AFM-Telethon

Ever since it was created AFM-Telethon has created or initiated many entities to serve the fight against disease.

Our partners

In therapeutic innovations

- **ACADEMIC**
  Bpifrance, Inserm, CNRS, AP-HP, CEA, Universities, Pasteur Institute, ANR, CHU (University Hospitals), EFS, Imagine Institute...
- **PATIENTS' ORGANISATIONS**
  IFCAH, Vaincre la Mucoviscidose, Retina France, Vaincre les Maladies Lysosomales...
- **INDUSTRY**
  Biotechs, international and national pharmaceutical groups
- **INTERNATIONAL NETWORKS**
  SMA Europe, Collagen 6 Alliance, EuroNMD, IRDiRC, COST Exon-skip, Cure CMD, EJP-RD...

In social, medical and technological innovation

- **INDUSTRY**
- **NON-PROFIT ORGANISATIONS,** including in French overseas departments and territories
- **MULTI-DISCIPLINARY CONSULTATION NETWORKS**

For Telethon

- **FRANCE TÉLÉVISIONS GROUP,** producer of the TV broadcast
- **MORE THAN 100 NATIONAL PARTNERS:** companies, professional and sport federations and non-profit organisations

Cure

Because neuromuscular disorders are rare diseases, and mainly caused by genetic mutations, AFM-Telethon conducts a strategy of innovation which benefits rare diseases at large. It initiated a new medicine that is emerging and spreading far beyond rare diseases.

ACCELERATING RESEARCH THANKS TO A UNIQUE STRIKE FORCE

— **The Biotherapies Institute for rare diseases:** the institute combines three leading laboratories in innovative treatments for rare diseases, which the AFM-Telethon has created:

— **Institute of Myology, Genethon and CECS/I-Stem**. The aim of the institute's 600 experts: accelerating the development of treatment for patients.

— **The Foundation for Rare Diseases:** AFM-Telethon is one of the founding members and the main financer of this French scientific cooperation foundation which aims at coordinating skills and creating synergies in order to promote the development of new therapies. Since its creation, more than 600 projects have been supported.

— **Imagine Institute:** AFM-Telethon is one of the founding members of the Imagine Institute, a research and care centre located within the Necker Paediatric Hospital in Paris. Its aim: to make diagnosis and treatment for genetic disorders available as quickly as possible.

— **A seed fund for innovative biotherapies and rare diseases**, created with Bpifrance, the French public investment bank. The fund finances start-up companies which are developing innovative therapies for rare diseases at a very early stage. 6 start-up companies have been supported since the creation of the seed fund.

DEVELOPING INNOVATIVE BIOTHERAPIES FOR THE BENEFIT OF THE GREATEST NUMBER OF PATIENTS

— **38 therapeutic trials in humans,** either on-going or in preparation, with the support of AFM-Telethon in 29 different diseases. These trials are carried out in rare diseases affecting the muscles, the brain, the liver, the immune system, the blood, the vision, the skin, the heart.... and mainly rely on innovative biotherapies: gene or cell therapy, pharmacogenetics, stem cell research...

AFM-Telethon aims at demonstrating the feasibility and efficacy of these treatments not only for neuromuscular diseases but also for rare genetic disorders,

models of more common diseases.
— **YposKesi**, an industrial gene therapy production facility set up by AFM-Telethon and Bpifrance, the French public investment bank, was reinforced in March 2021 by the arrival of an international industrial partner, the SK group. The aim is to have sufficient bioproduction capacities in France in order to meet the needs worldwilde of the increasing number of gene therapy projects and treatments and to accelerate the technological leap necessary in this field.
— **The development of tools and platforms to facilitate the organization of trials:** databases collecting patients' genetic and clinical data, clinical investigation centres...

SUPPORTING BOTH FUNDAMENTAL RESEARCH AND THE DEVELOPMENT OF INNOVATIVE THERAPIES
— **More than 350 scientific projects and young researchers funded** through calls for proposals, including 22 strategic projects and strategic research centres in France (MyOccitannie in Montpellier, Translamuscle in Créteil, MoThard in Marseille and MyoNeurALP2 in the Auvergne-Rhône-Alpes region).
— **Supporting other French partner associations in innovative biotherapy projects through their respective calls for proposal.** In 2022, AFM-Telethon funded six projects: two for Vaincre la Mucoviscidose (cystic fibrosis), two for Retina France (eye diseases), one for Vaincre les maladies lysosomales (lysosomales storage diseases), one for IRME (French Institute for Spinal Cord and Brain Research).

STIMULATING INTERNATIONAL CO-OPERATION
— **Participation in the European Reference Network (Euro-NMD) for neuromuscular diseases** which gathers 80 centres of expertise of 25 European countries. The Institute of Myology is one of the 10 centres of expertise located in France. AFM-Telethon is one of the patients' organisations represented in the governance of the ERN.
— **Participation in international research networks: IRDiRC** (International Rare Diseases Research Consortium) launched by the European Commission and the National Institutes of Health in the US in order to accelerate the development of medicinal products for rare diseases and to diagnose most of them; **EJP-RD** a European Joint Programme on Rare Diseases focused on research; **COST Exon-skip** a network dedicated to the development of exon skipping therapies
— **International associative partnerships: AFM-Téléthon** is developing research collaborations with **SMA-Europe** (spinal muscular atrophy), **the Collagen 6 Alliance** (congenital muscular dystrophies with collagen 6 deficiency) and **Cure CMD** (congenital muscular dystrophies).

CREATION OF GENOTHER

Spurred on by Genethon, the GenoTher biocluster was accredited in 2023 by the French National Research Agency, as part of the France 2030 plan. Dedicated to accelerating the development of gene therapy drugs, it brings together more than 35 partners from academia, industry, biotechs, investment funds, universities and grandes écoles around its seven founding members - Genethon, Spark Therapeutics (a Roche subsidiary), Genopole, AP-HP, Inserm, YposKesi and the University of Evry. Its aim is to make dozens of new gene therapies for rare and common diseases available over the next ten years, by attracting the best talent and speeding up the emergence of research and development projects. Public funding will amount to €70m over five years. This accreditation marks a major step in the creation of a European ecosystem to meet the scientific, technological and economic challenges associated with gene therapy. It lays the foundations for an industry of excellence that the AFM-Téléthon has been calling for for a long time to ensure France's health independence in the rapidly expanding field of genomic therapies.

Care

AFM-Telethon's action is varied, from access to diagnosis and adequate care to local support services. It is always looking for innovative solutions that meet the needs of patients and their relatives with a unique goal: help patients live their lives according to their own choices.

IMPROVING CARE PATHWAYS

— **Support for the network of specialist consultations and centres of reference throughout France.** In 2023, the AFM-Téléthon supported 48 multidisciplinary consultations.
— **Participation in Filnemus,** the French neuromuscular clinical network that unites all the experts involved in research, diagnosis, care and treatment of patients.
— **Abroad, support for associations that help to facilitate access to appropriate treatment for patients:** Tierno and Mariam's International Foundation (FITIMA) in Burkina Faso, and ALAN Maladies Rares in Luxembourg.

SUPPORTING PATIENTS AND THEIR FAMILIES

— **Local advocacy ambassadors called "departmental delegations":** volunteers affected by the disease represent the organisation and advocate for patients affected by a neuromuscular condition within local representative bodies. They work closely with families, providing information, prevention, combating isolation and facilitating access to rights.
— **Interests groups** (peer support groups): patients and families, experts in the same disease, are working together to support other patients and to contribute to advances in research, diagnosis and care in collaboration with scientists and doctors.
— **AFM-Telethon's 16 local branches called "Regional services"** where professionals support patients throughout the different stages of the disease (diagnosis, healthcare) and help them achieve their life goals (personal assistance, housing adaptations, technical aids, education, employment...). The "Référents parcours de santé" (integration technicians or facilitators) are unique professionals who act a intermediaries between the family and various health and social care professionals (neuromuscular consultations, local carers, MDPH, schools, etc.) and ensure that patients get the response that is tailored to their specific needs. In 2023, 7,783 patients and their

families were supported by the Regional Services.
— **A dedicated 24/7 helpline providing information and support** to people affected by a neuromuscular disorder and their relatives

STIMULATING SOCIAL AND TECHNICAL INNOVATION

— **Choosing a place to live:** AFM-Telethon runs a specialist residential care home in Saint-Georges-sur-Loire (France), and supported housing in Angers providing those who are heavily dependent with roundtheclock, 7 days-a-week emergency assistance in a council housing complex.
— **Supporting carers:** with the creation of the "Villages Répit Familles®" respite homes in Saint-Georges-sur-Loire

and Cotaux du Lizon (France) where families can stay to take a break with the support of trained professionals.
— **Facilitating independent life through technological innovation:** information and support to choose technological solutions (technical aids, domotics, information and communication technologies), collaboration with researchers and industry to develop technical devices that meet the needs of patients, failure and complaints observatory for wheelchair users, temporary lending of mobility devices...
— **Implementation of an action plan to promote shoulder and arm weakness compensation including the creation of specialist consultations in hospitals in Paris, Bordeaux, Marseille, Clermont-Ferrand** to give patients access to the relevant technical aid.

BRINGING PATIENTS' VOICES TO THE FOREFRONT
— **Foster the inclusion of people with disabilities into society** and reasserting the benefits of the February 11th 2005 French Disability Act. AFM-Telethon advocates for patients' rights in public authorities and national and local bodies. It also supports families, individually, to access their rights. In October 2023, faced with a shortage of human aid, AFM-Téléthon and APF France handicap referred the matter to the Défenseure des Droits (Human Rights Ombudsman) for endangering the lives of others and failing to provide general assistance to people in danger.
— **Guaranteeing early access to treatment and diagnosis for patients.** Following the revision of the French bioethics law to allow genetic screening at birth, AFM-Telethon continued its efforts to promote the extension of neonatal screening to spinal muscular atrophy, launching a trial in 2023 in two regions, Grand Est and Nouvelle Aquitaine. The Association has also been active in defending patient access to certain treatments.
— **Encourage and uphold a national policy for rare diseases.** AFM-Telethon contributed to the drafting of three national plans which were hailed as positive examples in other European countries. The Rare Diseases Platform is a single resources centre bringing together the main French and European players in the battle against rare diseases and is mainly funded by AFM-Telethon: the **Rare Diseases Alliance**, a French group of 240 patients' organisations; Eurordis, a European alliance of 1,000 rare disease patients' organisations from 74 countries; **Orphanet**, the European portal for rare diseases and orphan drugs (Inserm, a French public scientific and technological institute); **Rare Disease Info Service**, an information service helpline for health professionals and those affected; **French Foundation for Rare Diseases** (see page 6).

Communication

PROMOTING MUSCLE AS A PUBLIC HEALTH ISSUE

As part of the Association's statutes, communication helps to disseminate knowledge about advances in research to families and professionals, as well as to the general public, through a wide range of publications. In 2023, the AFM-Telethon and the Institute of Myology have launched a major communication and awareness-raising campaign focusing on muscle, this little-known organ which is so essential to our general health. In addition to our mobility, muscle interacts with other organs and helps to combat widespread diseases such as diabetes, hypertension, cardiovascular and neurodegenerative diseases, cancer, etc. The campaign was based around two key events: the «Assises» for institutions and experts and «Muscle Week» for the general public.

Accounting for THE USE OF DONATIONS

Since the first Telethon, AFM-Telethon pledged to report transparently on its actions and the use of funds.

DETAILED FINANCIAL ACCOUNTS AVAILABLE TO ALL

Every year, AFM-Telethon publishes an annual and financial report (including financial statements such as balance sheet, operating statement, use of resources statement detailing the use of donations, property assets, remuneration policy...). It is widely distributed and available on its website. Answering donor's question is also part of AFM-Telethon's commitment to transparency. Consequently, a donor-dedicated phone line is

N°Cristal 09 69 36 37 47
APPEL NON SURTAXÉ

PERMANENT CONTROL

AFM-Telethon's accounts are certified by an external auditor. Mindful of the rigorous and efficient use of donations, AFM-Telethon has set up several external and internal audit procedures. In addition, it is certified by Bureau Veritas, an independent body, since 2001*. AFM-Telethon is one of the most controlled French charities, whether by the French public authorities (3 audits from the Cour des comptes, the National Court of Auditors) or on its own initiative (Bureau Veritas Certification since 2001).

A VALUATION STRATEGY FOR THE BENEFIT OF PATIENTS

AFM-Telethon's research strategy has relied on funding therapeutic innovation for many years now, including through private sector partnerships. AFM-Telethon's primary objective when funding promising projects, whether public or private, is to ensure that promising projects are brought to fruition and result in medicines that are made available to patients at a fair and sustainable price, making them accessible to all. A secondary point is to guarantee a fair financial return so that drugs developed through Telethon-funded research will generate revenue once on the market so that they can be reinvested in the organisation's missions.
The main principles of this valuation policy were defined in 2004 by AFM-Telethon's Board of Trustees and are implemented contractually.

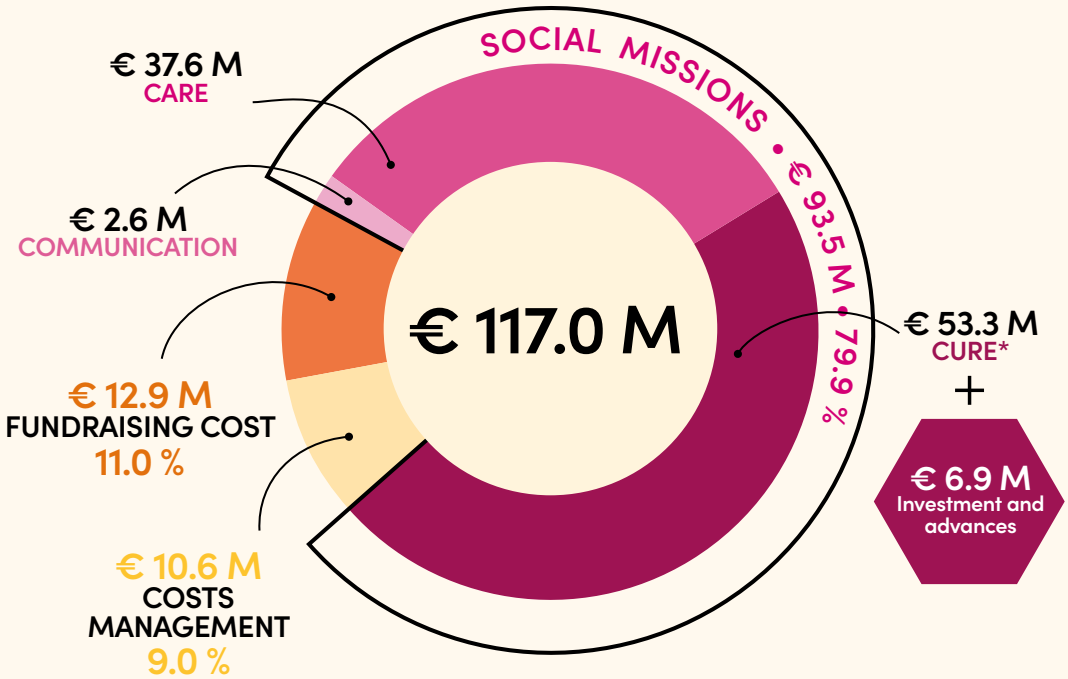
FOCUS ON THE USE OF DONATIONS

In 2023, the **€ 85.4 million** spent on our missions came from donations, amongst which:
- **€ 78.5 million** of expenses,
- **€ 6.9 million** of investment and advances for the cure mission.

Since 2001, the AFM-Téléthon has been committed to a voluntary process of transparency through certification by an independent organization, Bureau Véritas Certification. This certificate is issued following rigorous controls, guaranteeing that donations are used in accordance with stated objectives. The characteristics of this certification are as follows: 1- The AFM-Téléthon uses its resources to act in accordance with the missions it has set itself and which are known to donors. 2- The AFM-Téléthon's operations are guaranteed by defining responsibilities and practices. 3- Donors' rights are defined and respected, and the information provided to them is sincere. 4- The information transmitted by the AFM-Téléthon is transparent and consistent. Following the audit carried out at the end of 2023, beginning of 2024, AFM-Téléthon has obtained a 3-year renewal of its certificate.

— 2023 KEYS FIGURES

AFM-TELETHON'S Activities in 2023 all fundings included



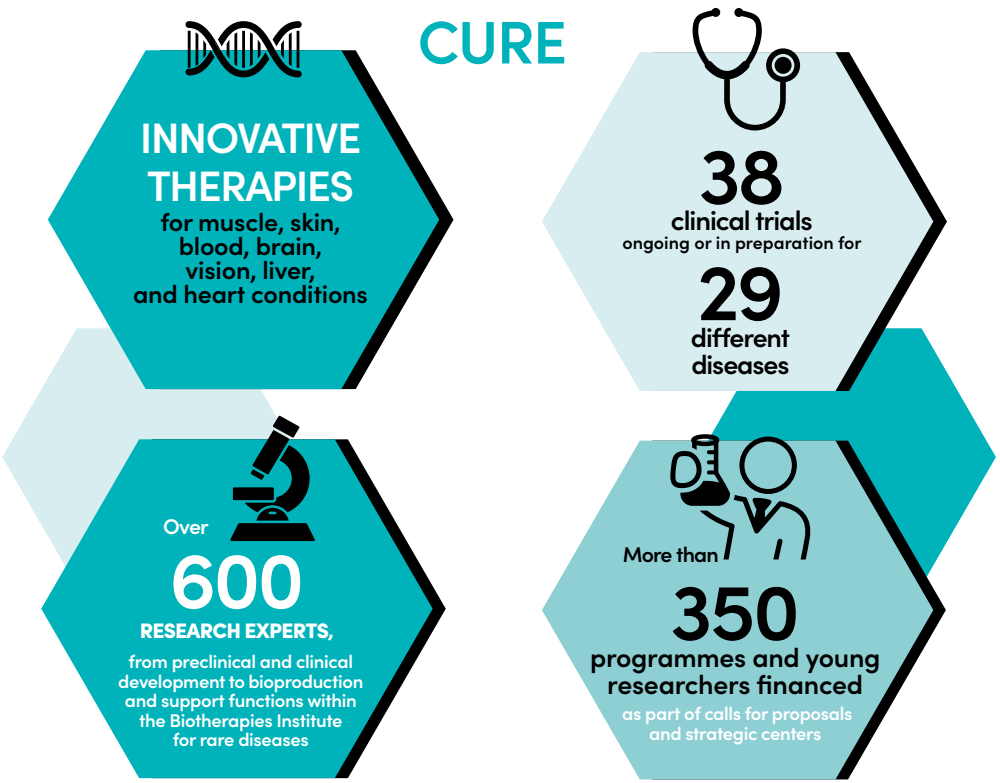
More information about our key figures, ressources and the use of donations in 2023 in our Annual Report on www.afm-telethon.fr (in French)

— 2023 IN BRIEF

€ 53.3 M committed to the **CURE** mission

+

€ 6.9 M of investment and advances



€ 37.6 M

committed to the **CARE** mission

CARE

2
Villages Répit Familles
(respite homes)

674
persons hosted

1
RARE DISEASES PLATEFORM
representing
6
main players in France and in Europe

181
professionals
in
16
regional services dedicated to family support

Telethon 2023

€ 92,905,533
raised

Close to
280 000
volunteers
mobilised to organise fundraising actions

More than
100
national partners

A **30**-hour
fundraising broadcast on France Televisions channels

More than
430,000
persons
follow social media accounts

65 years OF FIGHT
WHICH MARKED A SEA CHANGE

— The genetic revolution

From genome mapping to the discovery of genes responsible for diseases, the landscape has changed drastically. Thousands of families affected by genetic disorders now have access to diagnosis, genetic consultation, prenatal and pre-implantation diagnosis in order to make informed decisions about family planning.

— The social revolution

Thanks to the Telethon, the Organisation's 30-hour fundraising TV programme, citizens became involved in research and patients are now considered as partners by researchers and physicians. The general view of life with a disease and a disability has changed.

— The biotherapies revolution

Gene therapy, pharmacogenetics, stem cells: innovative therapies supported by AFM-Telethon are set to revolutionize the future of medicine. Patients affected by life-threatening immunodeficiency, rare blood diseases, brain or neuromuscular disorders can now benefit from the first research results.

1958

1958
Creation of AFM
Yolaine de Kepper, mother of 7 children including 4 boys suffering from Duchenne Muscular Dystrophy, set up the French organization for muscular dystrophy in Angers (France).

1969
Healthcare expenses related to muscle-wasting conditions finally benefit from the National Healthcare system funding,
the beginning of their recognition.

1972
AFM imports **the first electric wheelchairs in France** and fights restlessly for their funding by the French healthcare system (1977).

1981
Creation of AFM's first scientific board, the beginning of an unprecedented partnership between patients, researchers and physicians.

1987

1987
First Telethon on Antenne 2 channel (French Public Television). More than 181 million francs were raised (€27.6 million).

1988
Creation of the Regional services (AFM-Telethon's local branches) and **a new occupation, unique professionals who support patients and their families** throughout the different stages of the disease.

1990
Creation of Genethon today one of the world's leading laboratories researching gene therapy for rare diseases.

1992-1996
The publication by Genethon of **the first human genome maps** was hailed by the international scientific community, which marked the starting point for sequencing the entire human genome which was completed in 2003.

1996
Creation of the Institute of Myology, a centre of expertise for muscle and its diseases.

2000
First major therapeutic victory the bubble babies with immune disorders are successfully treated by gene therapy.

2001
Creation of the **Rare Diseases Platform** bringing together the six main French and European players in the fight against rare diseases.

2005
Creation of I-Stem, the spearhead of stem cell research.

2009
- Gene therapy proved its efficacy for adrenoleukodistrophy, a rare brain disease and beta-thalassemia, a rare blood disease.
- I-Stem succeeded in reconstructing an epidermis with stem cells.
- Opening of the very first **Village Répit Familles** (respite home), in St-Georges-sur-Loire, followed by the opening in 2013 of the Cizes facility.

2012
AFM-Telethon launched the Biotherapies Institute for rare diseases to accelerate the development of treatments.

2000

2013
Genethon obtained the authorization of producing innovative therapies from the French national medicines security agency.

2015
- **First graft of stem cells in the heart,** a world premiere.
- **New success** of gene therapy **for an immunodeficiency** (Wiskott-Aldrich syndrome).
- **Opening of I-Motion Institute** a unique platform dedicated to trials for children affected by neuromuscular conditions.

2016

2016
Setting up of YposKesi, an industrial facility dedicated to the development and production of innovative therapies.

2019

2019
A historical breakthrough for neuromuscular disorders. The first gene therapy medicine, using results achieved at Genethon, was approved for use in the United States for spinal muscular atrophy. In 2020 it is authorized in Europe and Japan.

AFMTÉLÉTHON
CURE THROUGH INNOVATION

A registered charity

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