AFMTÉLÉTHON
INNOVER POUR GUÉRIR

RENDRE
COMPTE

CURE THROUGH INNOVATION

JULY
2022

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 genetic condition 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

Research and development of therapies for muscle wasting conditions is the primary aim of AFM-Telethon. It chose to support research which benefits rare diseases at large, and even beyond. AFM-Telethon set up its own laboratories and means to accelerate the understanding of the mechanisms of the diseases, the development of innovative therapies based on the knowledge of genes and cells in order to make cures available to patients. Innovation and therapeutical efficacy are at the heart of its action.

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

GENOSAFE

Service provider
control and safety of biotherapeutic products

ATAMYO THERAPEUTICS

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

YPOSKESI

An industrial pharmaceutical facility dedicated to the development and production of gene therapies created with Bpifrance, the French public investment bank

SEED FUND

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

GENOPOLE® EVRY

research campus, centre of excellence, in genome and post-genomic studies

A PATIENTS AND FAMILIES ORGANIZATION

AFMTELETHON
INNOVER POUR GUÉRIR

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

Voluntary 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)

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

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

Patients' location to live in France
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
148 local teams of volunteers which coordinate the development of fundraising events during the Telethon in each French department

THE RARE DISEASES PLATFORM

A unique resource centre for rare diseases

Rare diseases alliance Eurordis
Rare diseases info service
Orphanet
Rare diseases foundation

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, DEBRA France...
- **INDUSTRY**
  Biotechs, international and national pharmaceutical groups
- **INTERNATIONAL NETWORKS**
  SMA Europe, Alliance collagène VI, EuroNMD, IRDiRC, COST Exon-skip

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
- **CLOSE TO 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 or largely finances: **Institute of Myology, Genethon and I-Stem**. The aim of the institute's 500 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, 400 projects have been supported.

— **Imagine :** 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. 7 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

— **40 therapeutic trials in humans,** either on-going or in preparation, with the support of AFM-Telethon. These trials are largely based 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 affecting the skin, the blood, the vision, the brain or the liver.

— **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 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 200 scientific projects and young researchers funded** through calls for proposals, including 20 strategic projects and 3 strategic research centres in France (Translamuscle in Créteil, MNH-Decrypt in Marseille and MyoNeurAlp in the Auvergne-Rhône-Alpes region).

— **Supporting other French patients' organisations in innovative biotherapy projects through their respective calls for proposal.** In 2021, AFM-Telethon funded five projects: two for Vaincre la Mucoviscidose (cystic fibrosis), one for Retina France (eye diseases), one for IRME (brain and spinal cord research), and one for IFCAH (endocrine system diseases).

STIMULATING INTERNATIONAL CO-OPERATION

— **Participation in the European Reference Network (Euro-NMD) for neuromuscular diseases** which gathers 84 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; **ENMC** (European neuromuscular centre), an internal network of researchers and clinicians to facilitate the research of treatments, to improve diagnosis and to optimize standards of care.

— **International cooperation with other organisations: SMA-Europe Europe** (muscular atrophy); **Collagen VI Alliance** (collagen VI deficiency congenital muscular dystrophies), and **Cure CMD** (congenital muscular dystrophy).

GENETHON'S RESEARCH LEADS TO MORE AND MORE CLINICAL TRIALS

Since 2020, close to 2000 children worldwide have been treated with the first gene therapy authorized for a neuromuscular condition, spinal muscular atrophy, a treatment based on technologies developed by Genethon. In 2021, two gene therapy trials developed by AFM-Telethon's laboratory started: a trial for Duchenne muscular dystrophy, led by Genethon, and a trial for Pomp disease led by Spark Therapeutics. In addition, Atamyo Therapeutics, a biotechnological company created by Genethon to accelerate the development of gene therapy for Limb grindle muscular dystrophies (LGMD), obtained approval to start a first gene therapy trial for FKRP linked LGMD in France, Denmark and the United Kingdom. The fruit of 30 years of research, at Genethon, conducted by Isabelle Richard, an international expert of these diseases. In total, 12 products developed by Genethon, alone or in collaboration with partners are now undergoing clinical trials throughout the world and seven others are in preparation.

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 HEALTHCARE

— **Support the neuromuscular consultation and centres of reference network throughout France** where patients can see specialist healthcare professionals at one and the same venue.

— **Participation in Filnemus,** the French neuromuscular clinical network that unites all the experts involved in research, diagnosis, and healthcare.

— **Conducting working groups focused on health and medical issues** (respiration, cardiology, pneumology, pain relief, orthopaedics...). The aim is to update, harmonise and improve health care practices continuously.

— **Support organisations contributing to improve medical care for patients abroad:** Tierno and Mariam's International Foundation (FITIMA) in Burkina Faso, West African reference network for muscular dystrophies (ROAMY) 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 also provide patients and families with support and advice.

— **Interests groups:** patients and relatives affected by a neuromuscular disorder meet with researchers and clinicians in 9 committees (each of them is dedicated to a specific condition). They support their fellow patients and take part in research, medicine development and healthcare processes.

— **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...). These unique professionals act as intermediaries between the family and various health

and social care professionals (neuromuscular consultations) and make sure that patients get appropriate care. 8,090 patients and their families received support from AFM-Telethon's Regional services in 2021.

— **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.

— **Guaranteeing early access to treatment and diagnosis for patients.** In 2021, following the change in French bioethics regulations authorizing newborn genetical testing, AFM-Telethon proceeded to promote the extension of newborn screening for spinal muscular atrophy (in June 2022, launch in cooperation with Filnemus of a pilot study programme in two French regions, Grand Est and Nouvelle Aquitaine). The organization also took action with Filnemus in order to guarantee patients early access to Covid-19 vaccination.

— **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 more than 200 patients' organisations; Eurordis, a European alliance of more than 900 rare disease patients' organisations from 72 countries; **Orphanet**, the European portal for rare diseases and orphan drugs; **Rare Disease Info Service**, an information service helpline for health professionals and those affected; the Rare disease Foundation (see page 6).

Communication

SHARING AND SPREADING KNOWLEDGE

The Organisation's mission statements specify that communication contributes to conveying to patients and their relatives, professionals and to the public at large up-to-date knowledge on neuromuscular conditions based on scientific research progress. AFM-Telethon produces several publications and arranges visits of the laboratories it funds throughout the year. Besides, AFM-Telethon organizes every year the "1,000 researchers in schools" programme intended for classes of students from junior to high schools. Researchers meet them in their classrooms and explain neuromuscular conditions as well as the latest research advances.

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 available, the donors' direct line:

0 825 07 90 95 Service 0,15 € / min + prix appel

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 (4 audits from the Cour des comptes, the National Court of Auditors) or on its own initiative (by IGAS, the French government audit office for social affairs, in 1989; by Arthur Andersen in 2000 and Bureau Veritas 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 make sure that they will lead to effective treatments for patients at a fair and affordable price for 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

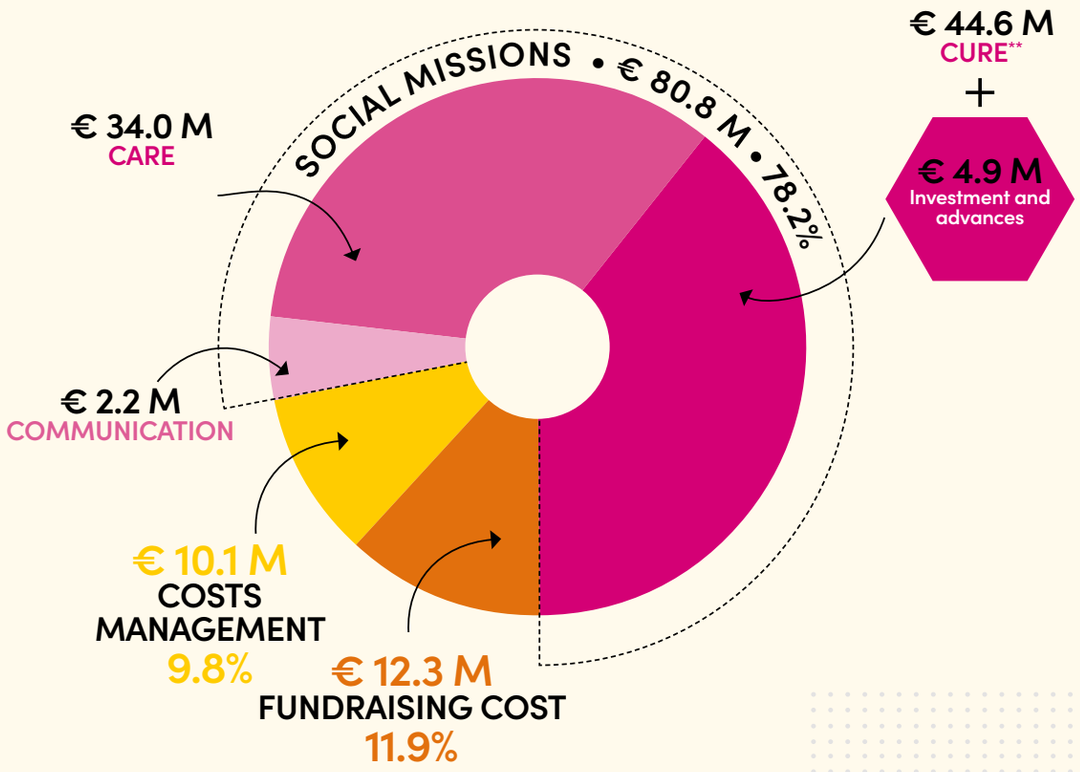
In 2021, the **€ 71.2 million** spent on our missions came from donations, amongst which:
- **€ 66.3 million** of expenses,
- **€ 4.9 million** of investment and advances for the cure mission.

* Following the audit between October 2020 and March 2021 by Bureau Veritas Certification (BVC), AFM-Telethon's certification was renewed for three years, it includes a follow-up every 18 months. This certification ensures that the services of the organisation comply with the following commitments: AFM-Telethon uses its resources to act in accordance with its mission statements which are known to donors; the operation of the AFM-Telethon is guaranteed by the definition of responsibilities and practices; donors' rights are defined and enforced; information given to them is truthful; information provided by AFM-Telethon is transparent and consistent.

— 2021 KEYS FIGURES

AFM-TELETHON'S Activities in 2021 all fundings included*

€ 103.3 M



* More information about our key figures, ressources and the use of donations in 2021, in our Annual report on www.afm-telethon.fr

** AFM-Telethon contributes to the financing of Genethon thanks to donations made at Telethon's fundraising events.

— 2021 IN BRIEF

€ 44.6 M committed to the **CURE** mission + **€ 4.9 M** of investment and advances

CURE

INNOVATIVE THERAPIES
for muscle, skin, blood, brain, vision, liver, and heart conditions

40 clinical trials ongoing or in preparation for **32** different diseases

Over **500** **RESEARCH EXPERTS,** from preclinical and clinical development to bioproduction and support functions within the Biotherapies Institute for rare diseases

More than **200** programmes and young researchers financed

€ 34.0 M committed to the **CARE** mission

CARE

2 Villages Répit Familles® (respite homes)
666 persons hosted

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

176 professionals in **16** regional services dedicated to family support

Telethon 2021

€ 85,933,166 raised

Close to **215 000** volunteers mobilised to organise fundraising actions

Close to **100** national partners

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

More than **400,000** persons follow AFM-Telethon social media accounts

64 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 muscles and their 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** the only resource centre for rare diseases in Europe.

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, both providing much needed respite for people with disabilities and their caregivers.

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.

AFMTÉLÉTHON
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A registered charity

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