RENDRE COMPTE

# CURE THROUGH INNOVATION

SEPTEMBER 2021

AFMTÉLÉTHON
CURE THROUGH INNOVATION

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.

AFMTÉLÉTHON
CURE THROUGH INNOVATION

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.

"Wonderful victories against disease are gained today thanks to everyone's support."

**LAURENCE TIENNOT-HERMENT**
PRESIDENT OF AFM-TELETHON

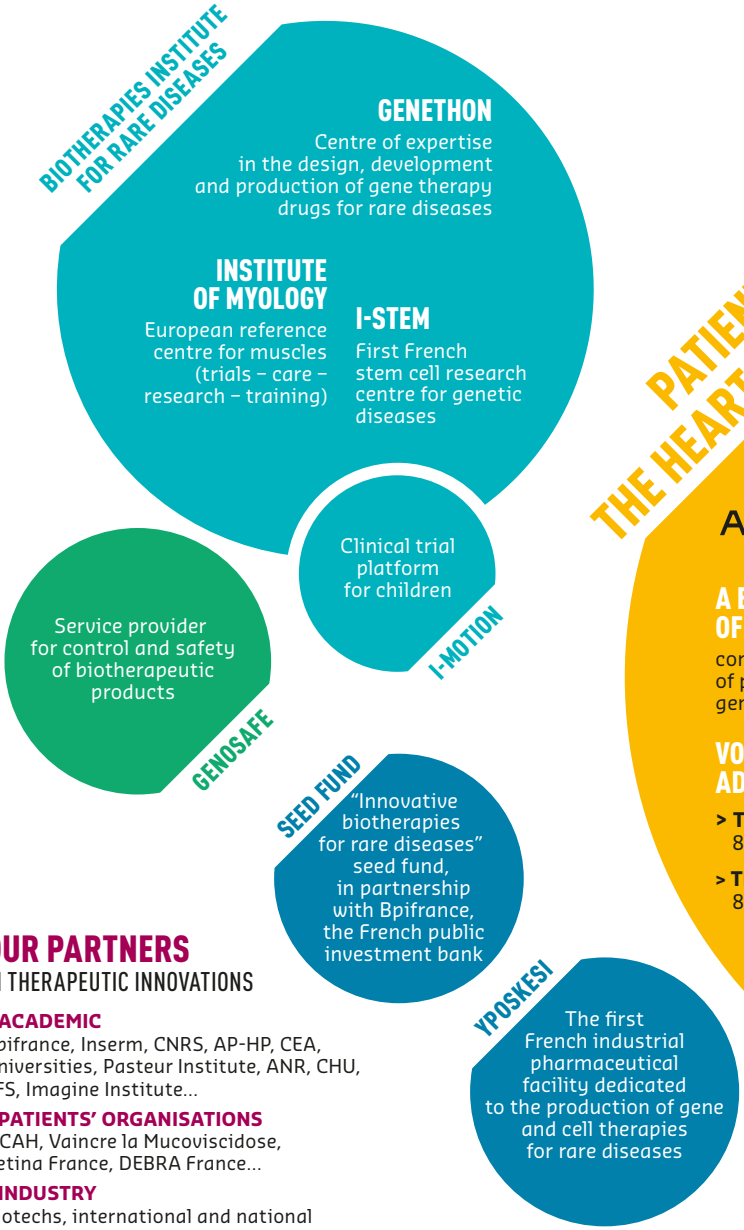
# THREE MISSIONS

## DEFINED BY THE ORGANISATION'S MISSION STATEMENTS:

**CURE** Research & development of therapies

**CARE** patients and their relatives

**COMMUNICATION** related to its missions




## OUR PARTNERS
IN THERAPEUTIC INNOVATIONS

- **ACADEMIC**
  Bpifrance, Inserm, CNRS, AP-HP, CEA, Universities, Pasteur Institute, ANR, CHU, 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, RD-Connect, COST Exon-skip
- **GENOPOLE® ÉVRY**
  Research campus, centre of excellence in genome and post-genomic studies

## PATIENTS AT THE HEART OF THE BATTLE

AFMTÉLÉTHON
CURE THROUGH INNOVATION

### A BOARD OF TRUSTEES
consisting of 20 patients and relatives of patients elected at the annual general meeting of members

### VOLUNTARY ADVISERS
> **The finance committee**
8 financial experts

> **The scientific council**
85 international experts

## OUR FAMILY NETWORKS

AFMTÉLÉTHON
CURE THROUGH INNOVATION

### 16 REGIONAL DEPARTMENTS

Teams of professionals who support the patients' life-project

### 69 DELEGATIONS

Volunteers affected by the disease to represent AFM-Telethon in the French departements

### 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**

**LE VILLAGE RÉPIT FAMILLES®**
**LES CIZES**

## OUR PARTNERS IN SOCIAL AND TECHNOLOGICAL INNOVATION

- Industry
- Non-profit organisations
- Multi-disciplinary consultation networks
- Non-profit organisations supported in French overseas departments and territories

## OPERATIONAL DEPARTMENTS

to implement the policy of the management board

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

## GENOCENTRE

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

## TELETHON

AFMTÉLÉTHON
CURE THROUGH INNOVATION

### 149 LOCAL TEAMS OF VOLUNTEERS

The teams of volunteers which coordinate the fundraising events during the Telethon in each French department

## OUR TELETHON PARTNERS

- France Télévisions Group, producer of the TV broadcast
- 96 national partners (companies, professional federations and non-profit organisations...)

## AFM PRODUCTIONS

Movies to spread knowledge and raise public awareness about the plight of patients

## THE RARE DISEASES PLATFORM

**A unique resource centre for rare diseases**

**RARE DISEASES ALLIANCE**
**EURORDIS**
**RARE DISEASES INFO SERVICE**
**ORPHANET**
**RARE DISEASES FOUNDATION**

# CURE

**Rare diseases, and especially muscle-wasting conditions, are entering a new era with the first innovative therapies made available for patients. In 2020, positive research outcomes continued to accelerate despite the Covid-19 health crisis that had an impact on research centres and teams. AFM-Telethon did everything in its power to limit the consequences of the crisis and to pursue research projects and clinical trials in an unprecedented context.**

## 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, 380 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

- **38 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 innovative medicine production facility** set up by AFM-Telethon and Bpifrance.
- **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 **15 strategic projects** and **3 strategic research centres** (multi-year funding): clinic-oriented projects; platforms and networks (pharmacological screening for mitochondrial diseases and ultra rare neuromuscular conditions…); 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 gene and cell therapy projects through their respective calls for proposal: Retina France and Gêniris (diseases affecting the vision), IRME (brain and spinal cord research), Vaincre les maladies lysosomales (lysosomal storage diseases), ARSEP (multiple sclerosis), Fondation du Rein (kidney diseases), Vaincre la Mucoviscidose, (cystic fibrosis), IFCAH (endocrine system diseases). In 2020, the organisation funded eight projects: two of Vaincre la Mucoviscidose, two of DEBRA France (epidermolysis bullosa), two of IFCAH and two of Retina France.

## **STIMULATING** INTERNATIONAL **CO-OPERATION**

- **Participation in the European Reference Network (Euro-NMD) for neuromuscular diseases created in 2016:** gathers 61 centres of expertise across Europe. The Institute of Myology is one of the 8 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; **COST Exon-skip,** an international network aiming at promoting exon skipping gene therapies.
- International cooperation with other organisations: **SMA-Europe,** to accelerate research and development of treatment for spinal muscular atrophy; **Collagen VI Alliance,** an international alliance to drive forward the development of treatments for collagen VI deficiency conditions.

### GENETHON, 30 YEARS OF INNOVATION THAT INITIATED A MEDICAL REVOLUTION, THAT OF GENE THERAPY

**In 2020, the first gene therapy treatment for a neuromuscular disorder, originating in Genethon's research, obtained market authorization in Europe. A symbolic milestone for the laboratory that celebrated its thirtieth birthday that very same year.**

Since its creation by AFM-Telethon in 1990, Genethon never stopped innovating. A pioneer of human genome mapping, of the identification of several hundreds of genes responsible for rare diseases and of gene therapy, the laboratory went through all the stages of medicine development. 2020 was a milestone for the laboratory that obtained the European Medicines Agency market authorization for spinal muscular atrophy type 1 gene therapy, derived from technologies developed at Genethon. The laboratory also obtained an agreement from French health authorities to start a gene therapy clinical trial for Duchenne muscular dystrophy. Today, ten Genethon products are at a clinical trial stage and eight are expected to start within five years. Thirty years of unrelenting research are bearing fruit.

# CARE

**Supporting patients and their families throughout the different stages of the disease, creating innovative solutions to meet their needs, advocating for the rights of patients and people with disabilities and preparing access to the first treatments, our actions are many and varied, with one objective in mind: facilitating the achievement of their life goals.**

## PROMOTING STATE OF THE ART HEALTHCARE TO IMPROVE PATIENTS' QUALITY OF LIFE

- **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 reference network that unites all the experts involved in research, diagnosis and healthcare.
- **Conducting working groups focused on health and medical issues** (respiration, cardiology, pneumology, ophtalmology, pain relief, orthopeady...). 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.

### PROTECTING PATIENTS AND THEIR FAMILIES FROM COVID-19

**In 2020, the health crisis emphasized the relevance of the support system created by AFM-Telethon.**

AFM-Telethon took action in many ways:

- Mobilization of AFM-Telethon's local branches called "Regional services", and volunteers acting as local advocacy ambassadors or involved in the organization's interest groups to support and inform patients and their relatives: 23,000 contacts were established by phone, mail or e-mail during the first lockdown, video calls were organised to replace home visits.
- Information was posted on a regular basis on our website, blogs and social media, and disseminated during online video conferences. The 24h/7 helpline staff levels were increased to answer patients' questions.
- Close cooperation with the neuromuscular medical consultations and the centres of reference's network was set up to ensure the continuation of medical care and the launch of a psychological support programme.
- Advocacy action was taken to obtain masks (500,000 masks were distributed at the height of the shortage), to make sure people with a neuromuscular condition could benefit from specific measures to protect them from exposure at work and to have priority access to vaccination.

- **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 in hospitals, local physicians, local authorities, schools) and make sure that patients get appropriate care. 16,400 patients and their families received support from AFM-Telethon's Regional services in 2020 amongst which 8,431 benefited from reinforced assistance.

- **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.
- **Collect families' needs in terms of innovative equipments or services** so that they are taken into account by the industry: cooperation with researchers and industrial companies and creation of a user panel involved in the launch and review of products and services.
- **Implementation of an action plan to promote shoulder and arm weakness compensation technical aids** so that patients can have access to a relevant solution covered by the French Social Security system.

## 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. AFM-Telethon is currently experimenting a patient-centred support approach, launched in the aftermath of the January 2016 Health Act. Since 2019, the Regional Health Authority of Corsica also experiments AFM-Telethon's individual support approach locally, targetting people with debilitating neurodegenerative conditions.
- **Guaranteeing early access to treatment and diagnosis for patients** (particularly the extension of newborn screening).
- **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).

# COMMU-NICATION

## 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.

# 2020 REPORT

**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**

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. Using an approach commonly used for the certification of company's products and services, Bureau Veritas makes sure AFM-Telethon's procedures and management meet specific criteria defined beforehand by a board of independent experts. This certification was renewed in 2020 for 3 years*.

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

The **€ 68.2 million** spent on our missions (see p. 11) include activities not financed through donations, especially activities at Yolaine de Kepper residential care home that are publicly funded

**€ 63.3 million** spent on our missions came from donations, amongst which:

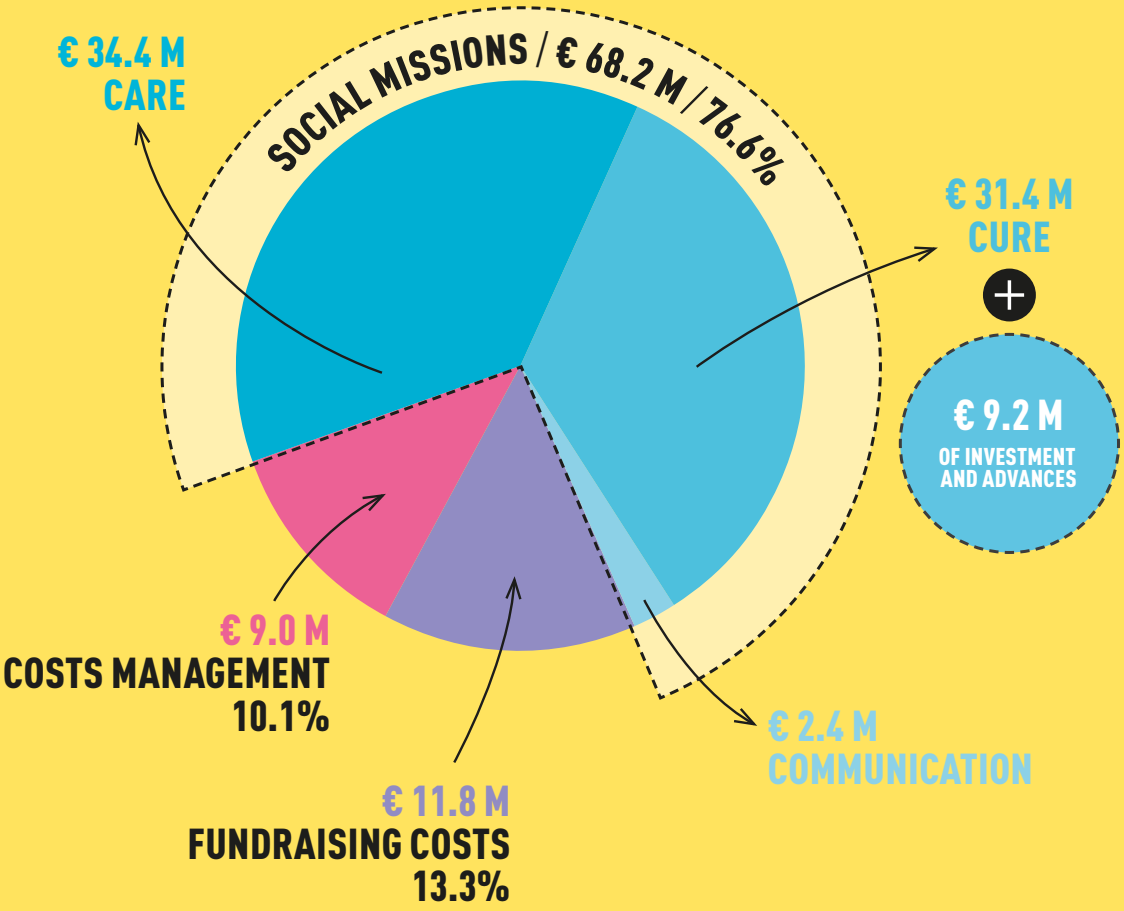
- **€ 54.1 million** of expenses,
- **€ 9.2 million** of investment and advances for the cure mission.

Organismes faisant appel à la générosité du public
VeriSelect CERTIFICATION DE SERVICES
Bureau Veritas Certification / 92400 Paris-la-Défense Cedex
Référentiel disponible sur demande

* 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.

## AFM-TELETHON'S ACTIVITIES IN 2020
ALL FUNDINGS INCLUDED

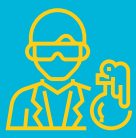
# € 89 M




Following the enforcement of a new Financial Accountability Act for non-profit organisations, AFM-Telethon has adapted its financial reports. The key figures it publishes come from the use of resources statement detailing the origin and use of funds, in addition to extra figures from the operating statement in order to report more extensively on the use of donations.

# IN BRIEF

**€ 31.4 M** COMMITTED IN 2020 TO THE **CURE MISSION** + **€ 9.2 M** OF INVESTMENT AND ADVANCES



**MORE THAN 200 PROGRAMMES AND YOUNG RESEARCHERS** FINANCED

**INNOVATIVE THERAPIES FOR**

MUSCLE, SKIN, BLOOD, BRAIN, VISION, LIVER, AND HEART CONDITIONS

**38 CLINICAL TRIALS** ONGOING OR IN PREPARATION FOR **31 DIFFERENT DISEASES**

**OVER 500 RESEARCH EXPERTS,** FROM PRECLINICAL AND CLINICAL DEVELOPMENT TO BIOPRODUCTION AND SUPPORT FUNCTIONS WITHIN THE BIOTHERAPIES INSTITUTE FOR RARE DISEASES

## GIFTS AND BEQUESTS

As a registered charity, AFM-Telethon is entitled to receive gifts, bequests and life assurance proceeds free of inheritance tax.

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relationstestateurs@afm-telethon.fr

## VOLUNTEERING

Get involved and put your energy and imagination in action to support patients.

benevoles@afm-telethon.fr

www.afm-telethon.fr/benevole

# € 34.4 M
COMMITTED IN 2020
TO THE
# CARE MISSION

**172**
PROFESSIONALS
IN
**16**
REGIONAL SERVICES
DEDICATED TO
FAMILY SUPPORT

**2**
VILLAGES RÉPIT FAMILLES®
(RESPITE HOMES)

**434**
PERSONS HOSTED
IN 2020

**1**
RARE DISEASES
PLATFORM
REPRESENTING
**6**
MAIN PLAYERS
IN FRANCE
AND IN EUROPE

## TELETHON 2020
# € 77,298,024
# RAISED

MORE THAN
**130,000**
VOLUNTEERS
MOBILISED TO ORGANISE
FUNDRAISING ACTIONS

**229,000**
FACEBOOK FANS

**145,000**
FOLLOWERS ON TWITTER
(INCLUDING ALL ACCOUNTS)

**3,214,703**
VISITS
ON THE AFM-TELETHON.FR
WEBSITE IN 2020

**96**
NATIONAL
PARTNERS

A **30**-HOUR
FUNDRAISING
BROADCAST
ON FRANCE TELEVISIONS
CHANNELS

# 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 expences 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.

# 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®**, in Saint-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.

# 1987

## FIRST TELETHON

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.

### 1992/96

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.

# 2016

## SETTING UP OF YPOSKESI, THE FIRST FRENCH INDUSTRIAL FACILITY DEDICATED TO THE DEVELOPMENT AND PRODUCTION OF GENE AND CELL THERAPY PRODUCTS

### 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** a unique platform dedicated to trials for children affected by neuromuscular conditions.

# 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.

# 60 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.

AFMTÉLÉTHON
CURE THROUGH INNOVATION

**A registered charity**

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Siège social: AFM-Téléthon - Institut de Myologie
47 - 83, boulevard de l'Hôpital - 75651 Paris cedex 13
www.afm-telethon.fr

Téléthon @telethon_france AFMTéléthon

Donors' direct line:

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

Neuromuscular patients' and families helpline:

0 800 35 36 37 Service & appel gratuits

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