

**Cure  
through  
innovation**

November 2013

**AFM TÉLÉTHON**  
CURE THROUGH INNOVATION



The VALUES  
of AFM-Téléthon  
are those  
held by the  
PATIENTS and their  
PARENTS who are  
DETERMINED  
to do everything  
it takes to  
CONQUER  
the disease

**WILLINGNESS  
AND CONVICTION**  
to make a choice

Since it was created, AFM-Téléthon does all it can to accomplish its goals and its conviction that a cure is possible.

**TRANSPARENCY**  
in actions

Transparency is more than a requirement – it is an ethical means. Since its first Téléthon, the Association has undertaken to provide its donors with a faithful account of the use of funds.

**REVOLT**  
against neglect  
and ignorance

Founding values of AFM-Téléthon – the revolt by parents who refuse to give in to misfortune and resignation.

**RIGOR  
AND EFFICIENCY**  
to succeed

This is the golden rule that AFM-Téléthon follows to achieve its targets, because it works in complex areas with limited resources.

Extract from the AFM charter, validated by the governing board on October 6<sup>th</sup> 2001.

- **An association of activists,**

patients and parents of patients committed to fighting rare genetic diseases which kill muscle after muscle – neuromuscular diseases

- **An organization combining volunteers and paid staff**

guided purely by the interests of the patients and the urgency of the progressive diseases

- **Three missions**

Defined by statutes of the Association:

- **Cure**

(Research & development of therapies)

- **Help**

(patients and their families)

- **Communicate**

(communication related to social missions/aims)

- **A goal**

which remains unchanged: to beat the disease

- **A strategy of general interest**

which gives priority to boldness and innovation to eradicate all rare diseases and benefit the disabled

- **An exceptional popular momentum through the Téléthon**

“

**THE MAJOR ACHIEVEMENTS, WHICH WE INSTIGATED ARE THE RESULT OF THE VITAL ASSOCIATION BETWEEN PATIENTS, VOLUNTEERS AND DONORS. THIS ASSOCIATION IS THE BACKBONE OF OUR CAMPAIGN AND OF OUR PAST AND FUTURE VICTORIES.**

”

**LAURENCE TIENNOT-HERMENT,**  
PRESIDENT OF AFM-TÉLÉTHON

## CURE

### BIOETHERAPIES INSTITUTE

#### GENETHON

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

#### INSTITUTE OF MYOLOGY

European reference centre for muscles (tests – nursing care – research – training).

#### I-STEM

First French stem cell research centre for genetic diseases.

#### ATLANTIC GENE THERAPIES

Gene therapy for rare diseases.

#### GENOSAFE

Service provider for control and safety of biotherapeutic products.



### Our partners in therapeutic innovations

#### ACADEMIC

Inserm, CNRS, AP-HP, CEA, Universities, Institut Pasteur, GIS/ANR...

#### PARTNERS

Retina France, IRME, AIRG/Fondation du Rein (Kidney foundation), Vaincre la Mucoviscidose (Cure for CF), SMA Europe, ARSEP...

#### INDUSTRY

Biotechs, international and national pharmaceutical groups

#### INTERNATIONAL NETWORKS

Treat-NMD, NMD Chip, Myores, IRDIRC...

#### GENOPOLE® EVRY

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

## PATIENTS AT THE HEART OF THE BATTLE

**AFM TÉLÉTHON**  
CURE THROUGH INNOVATION



### A Board of Directors

consisting of patients and parents of patients elected at the annual general meeting of members

### Voluntary advisers

- **The finance committee**  
6 financial experts
- **The scientific council**  
119 international experts

### Operational departments

to implement the policy of the management board

- Scientific
- Actions for families
- Medical action
- General secretariat
  - Resources
  - Collective action
  - Communication

## HELP

### Our family networks

#### 25 REGIONAL DEPARTMENTS

Teams of professionals who support the patients' life-project.

#### 73 DELEGATIONS

Volunteers affected by the disease to represent AFM in the French departments.

#### 7 INTEREST GROUPS

Volunteers who are experts in their specific diseases.

#### SUPPORT ASSOCIATIONS IN OVERSEAS DEPARTMENTS



### Our partners in social and technological innovation

- Industry
- Associations
- Multi-disciplinary consultation networks



### Patients' location to live

#### THE YOLAINE DE KEPPEL CENTRE:

- A residential home for looking after and welcoming high-dependence patients.
- Gate-Argent – an innovative concept of accommodation and services.
- The family respite village of Salamandre.

#### 3 PLACES TO TAKE A BREAK:

The houses of Etiolles and La Hamonais and an apartment in Paris.

## TÉLÉTHON

### 145 local teams of volunteers

The teams of volunteers which coordinate the Téléthon organization in each French department.

### 20 000 activities

organized by individuals, associations, districts...

### More than 70 national partners

Federations, companies, associations, professional organisations...

## COMMUNICATE

### AFM PRODUCTIONS

To spread knowledge and raise public awareness about the plight of patients.

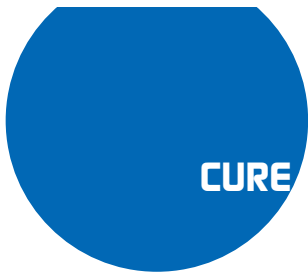
### GENOCENTRE

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

## THE RARE DISEASES PLATFORM

Unique resource centre for rare diseases

ALLIANCE OF RARE DISEASES  
EURORDIS  
RARE DISEASES INFO SERVICES  
ORPHANET  
INSTITUTE OF RARE DISEASES



**C**onvinced that innovation is the only means to cure the rare diseases which are still incurable, AFM-Téléthon plays a major role, both in France and internationally, in providing the impetus and development of innovative biotherapies that are vital for the rare diseases and are of benefit to medicine as a whole: the sole aim being to provide therapeutic efficacy for patients.



Genethon Bioprod: 5,000 m<sup>2</sup>, 4 production lines for gene therapy drugs

## Accelerate the cure thanks to a unique strike force

- **The Biotherapies Institute for rare diseases:** The Biotherapies Institute combines four leading laboratories in innovative treatments for rare diseases, which the AFM-Téléthon has created or largely finances: the Myology Institute, Genethon, I-Stem and Atlantic Gene Therapies. The aim: to accelerate the availability of treatment for patients.
- **The Fondation Maladies Rares (Foundation for Rare Diseases):** AFM-Téléthon is the founding member and main source of finance for this foundation of scientific co-operation, the aim of which is to combine skills and create synergies which encourage new treatments.
- **Imagine:** AFM-Téléthon is the founding member of the Imagine Institute, a research and care centre located within the Necker Hospital. Its aim: to discover and apply diagnostic and therapeutic solutions for genetic disorders as quickly as possible.

### / The Biotherapies Institute:

- 4 leading biotherapy laboratories: **Genethon** and **Atlantic Gene Therapies** for rare diseases; **The Myology Institute** for muscles and their disorders and **I-Stem** for stem cell therapy of genetic diseases
- More than 650 researchers, technicians, doctors, engineers, specialists in bio-production, clinical development or regulatory affairs...
- More than 25,000 m<sup>2</sup> of laboratories in Paris, Evry and Nantes

## ■ Developing innovative biotherapies to benefit as many as possible

- **36 therapeutic trials in humans, which are either on-going or in preparation**, with the support of AFM-Téléthon. These trials are largely based on innovative biotherapies: **gene or cell therapy, pharmacogenetics, stem cells...** with many emerging developments that the Association has been financing for many years and which are about to revolutionise medicine. As far as AFM-Téléthon is concerned, it is a matter of demonstrating the feasibility and efficacy of these treatments not only for neuromuscular diseases but also for rare genetic disorders, which are becoming more frequent. These clinical trials concern to roughly thirty different diseases (diseases of the blood, skin, muscles, vision, brain etc.).
- **Setting up production centres for genetic drug treatments for trials in humans:** Atlantic Bio-GMP in Nantes and essentially, **Genethon Bio Prod** in Evry, which has the largest facility in the world for producing gene therapy treatments.
- **Developing tools and platforms** to facilitate the organization of the trials: centres of clinical investigation, databases collating molecular and clinical data on patients...

## ■ Supporting fundamental research into the development of treatments

- **Supporting more than 300 scientific projects and young researchers** supported through the calls for proposals launched by the Association.

- **19 strategic projects supported** (several years' commitment exceeding 500 K€ per annum): clinical projects (gene therapy of Crigler-Najjar syndrome, gene therapy of Sanfilippo B syndrome, pharmacology of spinal muscular atrophy or alpha-sarcoglycanopathy...); strategic platforms or networks (modeling of synapses, development of non-viral vectors, pharmacological screening...).
- **Supporting associate partners** for gene and cellular therapy projects: diseases of the eye (**Rétina France**), the nervous system (**Institut pour la Recherche sur la Moelle Epinière et l'Encéphale**), the kidney (**Fondation du rein et Association pour l'Information et la Recherche sur les Maladies Rénales Génétiques**), multiple sclerosis (**ARSEP –Association pour la Recherche sur la Sclérose en Plaques**) and cystic fibrosis (**Vaincre la Mucoviscidose**).

## ■ Motivating international co-operation

- **Participation in international research networks:** **Alliance Treat-NMD** (European network of excellence (development of innovative treatments for neuromuscular diseases); **NMD-CHIP** European network (developing genetic diagnostic DNA chips for neuromuscular diseases); **IRDIRC** (International Rare Diseases Research Consortium) launched by the European Commission and the NIH (National Institutes of Health/USA), the aims of which are to finalise 200 new therapies by 2020 and to develop diagnostic tools for the majority of rare diseases; **Skip NMD**.
- **International associate partnerships:** **SMA Europe Alliance Treat-NMD, Skip NMD...** these 2 to be put above and keep DRCI (Duchenne Research Collaborative International...).



**B**y providing help to patients, as with its research, AFM-Téléthon focuses on innovation and is inventing concrete solutions to better meet patient requirements. Nursing care, providing close support, defending patients' interests... all the actions carried out by AFM-Téléthon in this sector have one and the same aim: to improve the patient's quality of life whilst awaiting a cure.

### ■ Improve patients' hopes and quality of life

- **Support the specialist consultation network** and reference centres, which allow the patients to meet all the specialists involved throughout the whole of France at one and the same venue.
- **Topic-related working group activities** (heart, respiration, pain, orthopaedics, occupational therapy...). The aim: to rationalise, harmonise and improve health care practices continuously.
- **Raising awareness and providing information about local care-givers** (local doctors, freelance physiotherapists...) concerning the specific nature of nursing neuromuscular diseases.

### ■ Providing daily family support

- **Departmental delegations:** volunteers affected by the disease represent the Association and bring their claims to the local authorities. They also provide the essential friendliness and a social link amongst those patients suffering from these diseases, who are frequently isolated.
- **Interest groups:** patients and families rally around to work together and contribute towards improving medical nursing care and speeding up research.
- **Regional departments:** professionals support people in drawing up and implementing their life-project throughout all stages of the disease: diagnosis, nursing care, education, employment, seeking human or technical assistance and so on. The implementing technicians act as an interface between the family and various professionals: neuromuscular consultations, local care-givers, the "MDPH" (local authorities for the disabled), educational establishments etc. And they are the guarantors of a response adapted to the specific nature of neuromuscular diseases.

### ■ Stimulating innovation

- **Choose a place to live:** creation of a **specialist residential facility** with enhanced care in Saint-Georges-sur-Loire (49) and a **"residential-services"** home in Angers (49), which provide round-the-clock security seven days a week for those who are heavily dependent and residing in a conventional public housing establishment.





## Speak up forcibly for the patients

- **Support for family care-givers:** development of the “**Family respite village**” concept, which enables families to avail themselves of these facilities, which have the expert nursing teams to cater for those who are heavily dependent. A first village was opened in 2009 in Saint-Georges-sur-Loire (49) for people suffering from neuromuscular and rare neurological diseases. A second village is under construction in the Jura region.
- **Development of innovative technical aids:** in partnership with industrial partners, AFM-Téléthon involves the users, right from the design phase of products up until their evaluation, whilst aiming to control costs and maintaining dynamism in the French market.
- **Strengthen the integration of the disabled into society and confirm the benefits of the law of February 11<sup>th</sup> 2005.** AFM-Téléthon upholds patient rights with the public authorities and national and local bodies (National Advisory Council of Disability, National Solidarity Fund for Autonomy, Authority for Health, MDPH...). It also supports the individual initiatives of families.
- **Encourage a national policy for rare diseases.** AFM-Téléthon contributed to the production of two national plans which were acknowledged as being true models by Europe. It also created and is the majority supporter of the **Rare Diseases Platform** – a single resources centre bringing together the main French and European players in the fight against rare diseases, i.e. the **Alliance Maladies Rares (Rare diseases alliance)** a French group of more than 200 patient associations; **Eurordis**, the European federation representing 561 associations from 51 countries; **Orphanet**, the European database of more than 6,000 rare diseases; **Maladies Rares Infos Service (Rare diseases – information service)** – information service for the health professionals and those parties involved; **La Fondation Maladies Rares (Rare diseases foundation)** (see page 4).

### / Sharing and spreading knowledge

The Association’s articles state that communication contributes to the diffusion of knowledge associated with the progress in research, to the families and the professionals, as well as to the public at large.

AFM-Téléthon has produced several publications intended both for the professionals and the families. It also organizes visits to the laboratories it finances throughout the year.

COMMUNICATING

# 1987 / 2012

## One fight... many results

### THE GENETIC REVOLUTION

The genome maps leading to the discovery of genes responsible for diseases represents an entire section of world medicine, which made a spectacular leap forward! Thousands of families affected by genetic disorders now have access to diagnosis, genetic consultation and prenatal and pre-implantation diagnosis in order to be able to expand the family circle, fully aware of the facts.

### THE SOCIAL REVOLUTION

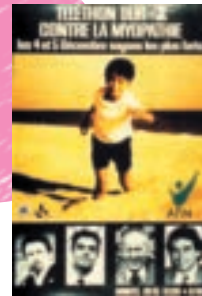
With the Téléthon, the citizens became the players in research and the patients the acknowledged partners of researchers and doctors. The general concept of the disease and disability has changed.

### THE BIOTHERAPY REVOLUTION

Gene therapy, pharmacogenetics, stem cells: all innovative forms of treatment, sponsored by AFM-Téléthon, have already revolutionised tomorrow's medicine and the lives of patients suffering from immunodeficiency, rare blood or brain disorders, who only a short while back, were sentenced without right of appeal and who can now benefit from the initial results of the research.

1987 →

1<sup>st</sup> Téléthon hosted by Claude Sérillon, Gérard Holtz, Jacques Chancel and Michel Drucker. The counter was a victim of its own success with more than 181 million francs raised (i.e. 27.6 million euros).



← 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 in Europe dealing with rare diseases.



↙ 2005

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

1990 ↓

Establishment of the Genethon laboratory, a real gene tracking factory.



↑ 1992-1996

Publication of the first human genome maps 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 excellence for muscles and their diseases.



↑ 2010

Yet another triumph! Thanks to gene therapy, a young patient suffering from  $\beta$ -thalassemia was able to resume a normal life, without having to rely on blood transfusions.

2009

Another victory! Two children suffering from a serious genetic disorder of the brain – adrenoleukodystrophy – undergo genetic treatment. The progression of their disease has stopped.



↖ 2009

I-Stem succeeded in reconstructing an epidermis with stem cells.

2012

AFM-Téléthon launches the Institute for Biotherapies for Rare Diseases

← 2011

AFM-Téléthon launches the production of gene therapy drugs to be tested in humans, with the help of the largest bio-production laboratory in the world, Genethon Bioprod.



# Statement of accounts 2012

**S**ince its first Téléthon, AFM-Téléthon has undertaken to give a transparent account of its actions and of the use of the funds entrusted to it.

## **Detailed accounts available to all**

Each year, AFM-Téléthon publishes an annual report showing the main activities of the past year together with the accounts (balance sheet, profit and loss statement, statement of employment of resources, real estate investment, salary policy etc.). This complete document, which is widely distributed, is accessible on the Internet or on request. The requirement for transparency pursued by AFM-Téléthon is also shown in the replies to the individual questions of donors. Since 2000, the association has installed its own direct donor telephone line, dedicated purely to donors.

## **Permanent controls**

Anxious to make rigorous and efficient use of the funds made available, AFM-Téléthon has set up numerous internal and external control procedures. Since 2001 it has been certified by an independent organisation – Bureau Véritas. Drawing its inspiration from an approach that is always open to companies by virtue of law No. 94-442 of 3<sup>rd</sup> June 1994, Bureau Véritas ensures conformity of procedures and the management of AFM-Téléthon by way of a reference standard that has previously been defined by a committee of independent experts. This certification was renewed in April 2011 for three years. AFM-Téléthon is therefore one of the most controlled associations in France, whether this relates to inspections ordered by the state (National Audit Office on two occasions) or on its own initiative (Igas in 1989, Arthur Andersen in 2000, Bureau Véritas since 2001).



Following its 2011 audit, AFM has obtained the renewal of its certificate for three years.

AFM-Téléthon is subject to Bureau Véritas Certification. This service certification ensures that the Association complies with the following commitments: AFM-Téléthon uses its resources to act in accordance with the tasks it has set itself and which are known to the donors; the operation of AFM-Téléthon is guaranteed by the definition of responsibilities and practices; the rights of the donors are defined and upheld; the information concerning them is true; the information issued by AFM-Téléthon is transparent and coherent.

# 100 €

Total activities  
for 2012:

120 M€

used by AFM-Téléthon in 2012 means:

## Missions\*

81.4 €

## CURE

### MAIN ACTIONS:

- The Biotherapies Institute for rare diseases: Genethon, Institute of Myology, I-Stem, Atlantic Gene Therapies
- Therapeutic trials
- Genethon Bioprod
- Calls for proposals
- International programmes
- Industrial programmes

## HELP

### MAIN ACTIONS:

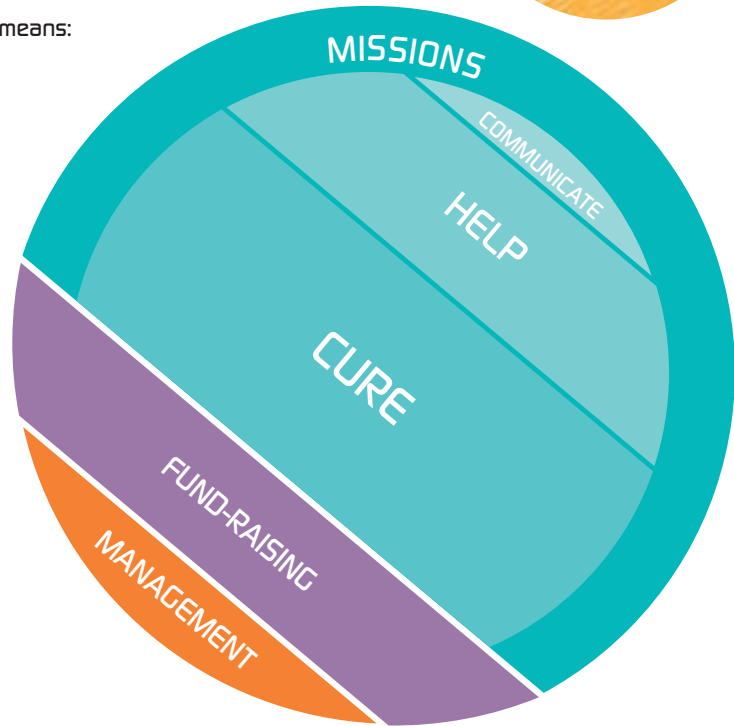
- Multi-disciplinary consultations
- Regional services
- Nursing care establishments
- Development of innovative technical aids
- Claims
- Family days
- Rare diseases platform

## COMMUNICATE

(communication associates with missions)

### MAIN ACTIONS:

- Genocentre conference venue
- VLM – the Association's journal
- Internet sites
- Editing information documents
- Relations with the media to release medical and scientific information
- Laboratory tours



## MANAGEMENT COSTS

7.3 €

- Management services
- Operation of internal bodies
- General meeting
- Financial communication

## FUND-RAISING COSTS

11.3 €

- Processing gifts and bequests
- Postage for pledges for gifts and tax returns
- Participation in the production of thirty hours of broadcasting
- Equipment and logistics of the 145 coordination teams
- Installing the 36 37 telephone donation line
- Setting up fund-raising on the web

\* The missions include the activities involved in providing care, which were financed to the tune of 7.2 million euros by social organizations. Excluding this amount, missions represent 80.2%. For the record, the commitment made at the time of the Téléthon 2011 was 81% - i.e. the same order of magnitude.



# The Téléthon, an exceptional event!



For the last 26 years, millions of persons have participated in the Téléthon at the end of the year, which is a festive and supportive event at the beginning of December. A strong and faithful engagement which makes it possible for AFM-Téléthon to continue its fight against the disease.



**30 hours**

direct on the channels of  
the France Televisions  
Group

More than  
**70** national partners  
(companies, professional  
federations...)



a **unique**  
television marathon  
in the world

**1,200** presenters,  
journalists, TV professionals  
mobilized





**200,000**  
volunteers

**20,000**  
animations

**5 millions**  
participants on D-day

# an unprecedented popular movement

**145** teams involved  
in departmental coordination;  
**1,800** volunteers called on  
throughout the year

## VOLUNTEER OPPORTUNITIES

To participate as a volunteer  
and use your energy and your  
imagination for the benefit of patients,  
contact:

**0800 695 501**

Toll-free number from a land line



**53,000**  
local associations  
and **10,000**  
committed districts

## BEQUESTS AND DONATIONS

**AFM-Téléthon**, as a recognised  
public association, is authorised  
to receive donations, bequests  
and life insurance, exempt from  
inheritance tax.

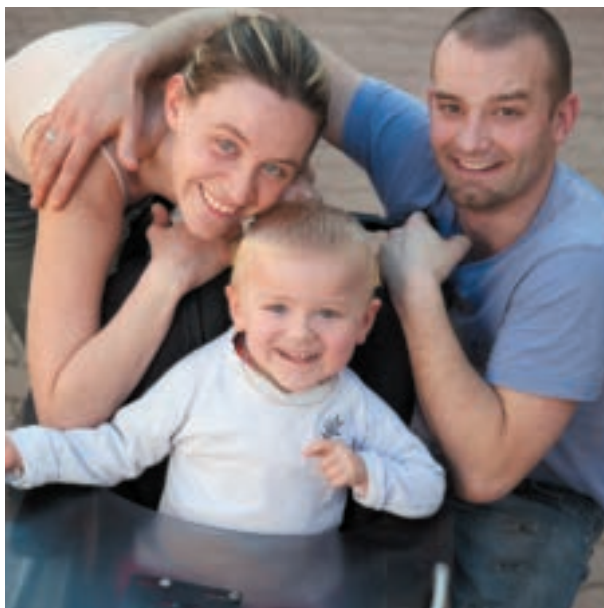
For any other questions, please  
contact: **33 (0) 1 69 47 28 13**



**1 million**  
donors

**149,000**  
fans on Facebook





A recognised public association

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