



OUR PARTNERSHIP PROJECT 2015-2020

Join our fight against rare diseases!

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1- A word from the chair

In the year 2000, forty organisations for people with rare diseases and their families, united by their shared experiences, decided to form an alliance. Today, French organisation *Alliance Maladies Rares* (the Rare Disease Alliance) can boast a network of more than 200 member organisations, which lend legitimacy to its work.

Some asked whether our optimism and the strength of our convictions would be enough to allow us to represent the three million people in France affected by rare diseases. The answer was yes! Despite the overwhelming daily struggles and the uncertain future faced by those with rare diseases, the hope that carries us and our work forward has always prevailed.

After fifteen years of taking action and working with public authorities, we can be sure that our constructive proposals have been a determining factor in rare diseases being taken into consideration in national health strategies.

Raising awareness among health professionals; making health care more structured, particularly through our *centres de référence* (referral centres), and more recently our *filiales de santé* (health networks); promoting research by establishing a *fondation de coopération scientifique* (foundation for scientific cooperation)... these are just some of the many successful initiatives led by the Alliance which have built on the solid foundations of our work.

But we want to go further, hand in hand with organisations interested in social or economic progress, which we hope will support us in the next phase of our development: our 2015-2020 strategic plan. Therefore, the idea came to us of inviting such organisations to join us on our journey towards a better world for the three million French people whose lives are hanging in the balance. Because all companies count among their ranks men and women affected either directly or indirectly by rare diseases.

We are counting on you to help us build a better world for them.

Alain Donnart

2- The Alliance's fight over the last 15 years

OUR MISSION STATEMENT

We are "a collective, a movement, a network", and we have three main missions:

- To increase knowledge and recognition of rare diseases among scientists, health professionals, public services, communities and the general public.
- To improve the quality of life and life expectancy of those with rare diseases by making it easier for them to access information, diagnosis, health care, their rights, treatment and support.
- To improve their chances of recovery through scientific and medical research.

Over the last 15 years, the Alliance has become the foremost representative of people affected by rare diseases in France. By working closely with both those affected by disease and the organisations that support them, the Alliance has been able to take their message to scientists, health professionals, health authorities and political authorities.

SPEAKING WITH A SINGLE VOICE

The Alliance has managed to bring people together and call them into action to give voice to needs that were previously ignored by public authorities.

Over our 15 years of existence, more than 200 organisations for rare diseases have sought out and joined the movement (representing more than 2,000 illnesses in France). One of the biggest achievements of the Rare Disease Alliance!

The Alliance has also made it possible to see beyond the particular circumstances of each disease and to focus on the experiences we share in order to be able to speak with a single voice and to put the spotlight on the obstacles faced by all people with diseases and their families. These obstacles can include inadequate treatment, daily difficulties in dealing with health professionals or authorities, or the many difficulties they must overcome to even get a diagnosis, which can sometimes take several years.

SEEKING TO INFLUENCE DECISION MAKERS IN TERMS OF HEALTH POLICY

The Alliance has made its voice heard in many influential arenas.

It is represented:

- at the *Ministère de la Santé* (Ministry of Health), through the national Rare Disease Plan
- at the *Agence Nationale de Sécurité des Médicaments*, which oversees the safety of medicines in France
- at the *Conseil National Consultatif des Personnes Handicapées*, a national council which is consulted on matters related to the application of French law giving disabled people equal rights and opportunities
- at the *Haute Autorité de Santé*, the high authority for health
- at the *Collectif Inter-associatif Sur la Santé*, a collective of organisations campaigning on health
- and at about ten other collectives and commissions.

IMPORTANT FACTS ABOUT RARE DISEASES

- A disease is said to be rare if it affects fewer than 1 in 2,000 people
- Between 7,000 and 8,000 rare diseases have been recorded up till now all over the world
- 3 million people are affected in France
- Rare diseases can affect movement (muscular dystrophies), sight (retinitis), hearing (Pendred syndrome), learning (fragile X syndrome), breathing (cystic fibrosis), resistance to infections, etc.
- 80% are genetic
- 65% are serious and incapacitating, and half result in shortened life expectancy.

KEY EVENTS OVER THE LAST 15 YEARS

- 1995: telephone helpline "Rare Disease Information Line" is established
- 24th February 2000: Rare Disease Alliances is established
- 2004: rare diseases become one of the five action areas for public health
- 11th February 2005: law protecting equal rights and opportunities for disabled people
- 29th February 2008: first international Rare Disease Day
- 2005-2008: first Rare Disease national plan -
131 rare disease referral centres and 502 official specialist centres open
- 2011-2014: second Rare Disease national plan -
- 2012: Rare Disease Foundation established
- 2014: Rare Disease health networks established

BEING A DRIVING FORCE FOR PROGRESS

Together, we have endeavoured to draw much more attention to what people with diseases and their families have to say and to their experiences, which previously had too often been ignored.

As evidence that this "expertise" is now more valued, their representatives were systematically included in the working groups that set up the first Rare Disease plan (2005-2008) and then the second plan (2011-2014).

But there remains much to be done, which is why the Rare Disease Alliance is campaigning for the large-scale actions undertaken in the second plan to be consolidated in the coming months, particularly the Rare Disease health networks, one of the most important aspects of the plan.

The Alliance has already started to campaign for a third national plan, which would go further on some matters such as the support given to people with rare diseases in their daily lives and in accessing healthcare, and the development of medical trials.

SUPPORTING OUR ORGANISATIONS

The Alliance offers a range of services to support and assist member organisations as well as individuals who are not part of an organisation. These services include: training (on listening, communication, etc.), information meetings providing up-to-date information on rare diseases, regional conferences, seminars, practical guides and legal advice.

Through its delegations, which can be found in each region and department of France, the Alliance is always there to listen to the needs of those with rare diseases and their families, as close to home as possible.

THE BOARD AND EMPLOYEES

21 representatives of Rare Disease Alliance member organisations sit on the national board.

The Alliance employs 7 highly-trained members of staff on a permanent basis.

3- Outlook for 2015-2020: our strategic plan

After 15 years of existence, the Rare Disease Alliance now wishes to take the next step in its development, by increasing its assets, prioritising some of its activities to better face current challenges related to rare diseases, and proposing new initiatives, involving both its members and its partners, in order to raise the profile and the impact of its fight. Its plan is structured around four main ambitions, which are related to both the Rare Disease Alliance's campaigns and its internal procedures.

AMBITION 1 - ADVANCING THE CAUSE AND IMPROVING HOW SOCIETY TREATS RARE DISEASES, THE PEOPLE SUFFERING FROM THEM AND THEIR FAMILIES

- Establishing rare diseases within the health system

They must remain a public health priority and should have a permanent place at the top of the national policy agenda. The Alliance is particularly keen to see a third national plan for rare diseases,

- Raising awareness about rare diseases

The greatest source of suffering for those affected by rare diseases and their families is often that the general public and health professionals have little or no knowledge of their disease, or even deny its existence. This is why spreading awareness and information is such a priority. Instead of doubting those who are affected, which can lead to false diagnoses, we want people to ask "could it be a rare disease?"

- Providing training on rare diseases

By definition, rare diseases require specialist knowledge and skills which are not provided by general training in medicine or in care, or are provided in a cursory manner. We must take action in this area so that the level of knowledge about rare diseases gradually reaches the same level as for other disabilities or chronic illnesses.

- Improving procedures for accessing healthcare

In almost every case, gaining access to a diagnosis, information, specialists in the disease, and any available treatment or care is an obstacle course for those affected by a rare disease and their families. We must act to change this state of affairs by increasing transparency and developing links between different medical, care and social professionals. Initiatives such as the rare disease health networks are designed to do this.

- Supporting and providing assistance to those affected by rare diseases and their families

The support and recognition currently given to carers, whether they are professional carers or family members, are inadequate. New support mechanisms and long-term care plans are essential for those affected by disease.

- Taking action in the field of medicine

Today, for the vast majority of rare diseases, there is no treatment, or treatment is limited to managing the symptoms of the disease. We must encourage research into treatments, and development, in order to put an end to this situation. We must also apply pressure for special medical uses of medicines in development to be permitted, based on the risk-benefit analysis for a particular patient, and with the full backing of member organisations.

- Promoting research processes which make room for those affected by rare diseases

We must continue the work we started with the creation of the rare disease foundation as part of the second national plan and promote research programmes launched by rare disease organisations. At the same time, we must campaign for rare disease organisations to be systematically included in research protocols and programmes from the beginning.

- Reforming society and defending the rights of those affected by rare disease

We must test out new educational and social methods designed to help those affected by rare diseases by putting an end to the isolation and even the discrimination that they experience all too often. Ensuring that those affected by rare diseases have the same rights as those not affected (information, credit, insurance, etc.) is a moral duty and gives us a new perspective on solidarity and on changes within our society.

AMBITION 2 - STRENGTHENING THE SENSE OF BELONGING, SUPPORT AND OPPORTUNITIES FOR DISCUSSION FOR THE ALLIANCE'S MEMBER ORGANISATIONS

- Improving training opportunities and opportunities to share information, experiences and good practice

The Alliance is planning to increase the number of national information and training sessions it holds on issues related to rare diseases and on good practice in terms of management and organisation for its member organisations.

- Providing more support for organisations' initiatives

The Alliance will provide more support for initiatives in the general interest carried out by member organisations in order to solve a problem or promote a proposal.

- Perfecting communication within the Alliance

Over the next five years, work to improve how we communicate with our member organisations (newsletter, website and social networks, face-to-face contact, etc.) will be carried out. Our existing procedures will be improved and new ones introduced.

- Providing more opportunities for organisations to meet through the Rare Disease March and the annual day of events for the Alliance's organisations

For 15 years, the Rare Disease March has brought together more than 2,000 marchers, including people affected by rare diseases and their families, every year in Paris. The Alliance will aim to further increase participation in this event, which raises money through a Telethon, and media coverage of it.

Introduced to mark the 15th birthday of the Rare Disease Alliance, the annual day of events for member organisations will see events taking place in every region and will coincide with the new event aimed at the general public, the Rare Disease Alliance's Extraordinary Regatta (see below), which will be launched in 2015.

AMBITION 3 - CONSOLIDATING THE ALLIANCE'S DEVELOPMENT AND REPUTATION

- Strengthening our local presence and action

More and more public health decisions are to be made at the regional level. The Alliance plans to reorganise its networks and its procedures in order to rise to this challenge which will affect those with rare diseases in their dealings with local health authorities. At the same time, it plans to strengthen ties and relationships with and between those affected by rare diseases, their families and local organisations, particularly for those which are most isolated.

- Increasing the Alliance's public presence in order to generate more support

For the first time in its history, the Alliance is preparing to launch an event which will allow it to inform the general public about its work and to call to action many new potential supporters.

This event, the Rare Disease Alliance's Extraordinary Regatta, will be held every year, on the second weekend in July, in both Paris and in other regions of France.

Fun races will be held, with original and wacky themes, allowing those who are affected by rare diseases and those who are not to meet each other and to come together in solidarity. These races will be organised by the Alliance in collaboration with aquatic sports clubs.

- Improving/developing our external communication methods

In line with the previous initiative, the Alliance will increase its involvement with the media, particularly social networks, allowing it to draw more attention to its work.

- Getting involved in international campaigns

The Alliance will lead a campaign for 2019 to be declared the European Year of Rare Diseases.

It will also take part in a major collaborative project bringing together people involved in the fight against rare diseases on both sides of the Mediterranean: this project will help those affected by rare diseases and their families living in north-west and sub-Saharan Africa to face the challenges of isolation and the lack of treatment and

support in these countries together. This project will be built on the principles of solidarity and of strengthening ties between Europe and Africa.

AMBITION 4 - PERFECTING THE ALLIANCE'S INTERNAL DYNAMICS AND WORKING METHODS

In the face of the challenges ahead, the five years of the strategic plan will also be the time to optimise our organisational structure and to reinforce the Alliance's most important resources, whether it's our commissions and working groups, our volunteers or employees, or our financial assets.

Particular emphasis will be placed on training our employees, elected members and appointed leaders; and on developing collaborative projects among the organisations that, alongside the Alliance, make up the Rare Diseases Platform.

4- Why support the Alliance?

MOVING IN THE RIGHT DIRECTION TOGETHER

TAKING ACTION to allow people who are often excluded and isolated to have the same opportunities and rights as any other citizen.

- one person in 20 is directly or indirectly affected by a rare disease. For example, if an organisation has 1,000 employees, this means that 50 people might be affected.
- through its action, which aims to relieve the burdens and difficulties of everyday life for those affected by rare diseases and their loved ones, the Alliance seeks to improve their quality of life and to allow them to achieve their full potential as a person, citizen, employee, etc.

SHARING our values and responsibilities

- giving a new meaning to corporate social responsibility
- sharing the values which are so important for team-building: refusing to give up, struggling on in the face of obstacles or severe difficulties, showing entrepreneurial spirit and taking risks, etc.

PROMOTING solidarity and the common good

- supporting the Alliance means taking a stand for solidarity in a society which leaves the weakest behind more and more often
- supporting the Alliance means supporting innovation and the search for new solutions in cases which are often so serious (and sometimes involve significant risks) that when progress is made, it can make a huge difference, even outside the field of rare diseases.

PROVIDING US WITH THE RESOURCES WE NEED TO IMPLEMENT OUR STRATEGIC PLAN FOR 2015-2020

The Alliance wishes to bring together 10 organisations, each one of which would make an annual contribution of 50,000 euros to the cause and would commit to supporting the Alliance over the five years of the strategic plan (an initial phase with a financial commitment over three years followed by a second phase of two years).

Over the last 15 years, the Rare Disease Alliance has enjoyed the support of the *AFM* (French Muscular Dystrophy Association) through its Telethon, the French Ministry of Health and other important partners such as: *Les Entreprises du Médicament* (an organisation representing pharmaceutical companies in France), *Fondation Groupama pour la Santé* (charitable foundation of a French insurance group), *Genzyme* and *Laboratoire Français du Fractionnement et des Biotechnologies* (biopharmaceutical companies specialising in treatment of rare diseases), and *Merck Serono* and *Shire* (international pharmaceutical companies).

5- What we have to offer...

We wish to raise support from a range of organisations, reflecting the values and the work of the Rare Disease Alliance in their entirety. If you can join us on our adventure, we can offer you:

AN EXCHANGE OF SKILLS AND KNOWLEDGE

- the chance to have your employees work alongside ours, preparing for the Alliance's events
- the chance to have the Alliance visit your organisation for an annual day to raise awareness and share information and ideas
- the chance to attend an annual debate on the current issues and challenges related to rare diseases, bringing together all our sponsors: the "Interview with the Alliance"

RAISING OUR PROFILES TOGETHER

- your support will be mentioned on institutional communication material in our list of partners ("The Rare Disease Alliance is supported by...") and your logo will be displayed on our website and brochures
- your support will be mentioned on publicity material for our events alongside your logo (Rare Disease Day, Rare Disease March, etc.)
- sponsors will, with approval from the Alliance, be able to publicise their support through their own institutional material using one of the following phrases:
 - "... supports the Rare Disease Alliance"
 - "... supports the Rare Disease Alliance, which is organising [project/event]"
 - "... is taking part in [project/event], organised by the Rare Disease Alliance", with a link to the Alliance's website.

Your contribution to our work will be firmly in the spotlight over the next five years!

6- TO JOIN US:

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