INTRODUCTION

- Personal experience
- First contact with the AFM

I – THE AIMS OF THE AFM

1) The patient becomes an expert on his disease
   - public panels
   - colloquiaums
   - training
   - interest groups: collecting data
     accumulating different observations
     production & diffusion of information
     dialogues
     visiting laboratories

- GRA

2) Alliance between researchers, clinicians and patients
   - CURE: make certain choices
     take risks
     attend to the most urgent needs
     collaboration
   - HELP: palliate certain disabilities

II – FUNCTIONING OF THE AFM

1) Board – executive power
   Commission – advisory power
2) Téléthon – financial independence

CONCLUSION

Our responsibilities
We are ALL concerned

INTRODUCTION

Good morning, ladies and gentlemen.
First of all, I would like to thank you for your invitation. I am very happy to be with you here and to speak to you about the AFM, an association of patients, and explain its objectives and the way it functions.
But briefly before that, I’d like to tell you about my own personal experience so that you will be able to measure the impact that an NGO can have on the life of a family whose children suffer from a rare disease.

I request your indulgence in advance for my English but I will, of course do my best.

In 1988, I gave birth to our first daughter. When she was nine days old, she was diagnosed with spinal muscular dystrophy. She passed away 3 months later. At the time, there was no information, no research on this disease and there was certainly no question of genetic testing. We were not ready to give up the idea of having our own children, so we decided to take the risk and start again. By that time we knew that we had 3 out of 4 chances of having a healthy baby. At the end of the year 1989, I gave birth to my second daughter but unfortunately she too had the same disease. She passed away 5 months later. After two such unhappy experiences, we were no longer prepared to trust in our luck.

It was at that time that I made contact with the AFM. I met people there who gave me the information I had been looking for, for such a long time, I met with people who listened to me, comforted me .... people who gave me hope in the future once again.

I was lucky enough to have the opportunity to meet a researcher who was prepared to spend the necessary time on researching this disease but lacked the funds. With the help of the AFM and the funds raised by the Téléthon, we found the money. The gene was localized 6 months later!!

The fact that its localization was now known meant that we were able to benefit from prenatal diagnosis. Once again, we were able to look to the future...

My three following pregnancies were without any problem and we have today 3 perfect girls.

I will never forget that the access to this genetic test enabled me to rebuild my family.

For the last 13 years, I’ve been a voluntary worker for the AFM. I have taken part in the SMA Interest group, worked with the Board of Directors and on different commissions. The AFM has become an integral part of my life and has given a sense to it.

So now let’s get to the heart of the matter which is, of course, the aims of the AFM.

1) One of the principal aims of the AFM is to enable the patient to become sufficiently knowledgeable about his or her disease even, if you like, to become an expert on the subject.

It is with this idea in mind that the AFM has set up different activities such as conferences, poster campaigns, colloquiums, training sessions, interest groups as well as “public panels” which Martine will go into in more detail later. But first of all, I would like to explain a little more about the functioning of the interest groups.

Within the AFM, there are several interest groups which exist. I would like to speak about one in particular: the SMA where I have been active over
the last ten years. Its activity to my mind is particularly pertinent. In this group, we record real-life experiences and collect as much information as possible. The families also play an active role in the production of information which as a result leads to a very comprehensive study of the disease (lesion) and its consequences (handicap) – the disease becomes visible.

Regularly, the families can go and visit the laboratories, ask questions and exchange information with the researchers giving a real sense to the work carried out in the laboratories.

Other work groups on pathology called GRA – reflection and action groups - meet four times a year in order to take stock of the evolutions of the pathology. The group is made up of researchers, clinicians and two families representing patients. These 8 groups concerning different diseases have been in place for two years now and have shown promising results.

2) The AFM also has the aim to encourage constant contact between researchers, clinicians and patients. Let’s not forget that the principal priority of the Association is to CURE the patient: for an association for a disease considered rare and often incurable, the aim is ambitious! But at the AFM, we always want to go further, do always more, and always go quicker. We want to fight this enemy which is destroying our children. It’s a constant obsession!

Therefore, science itself is at the heart of our reflection. We have to make choices, take risks and not be influenced by passing trends, to manage the more urgent needs. With these priorities in mind, we want to establish a real collaboration between the patients, the clinicians and the researchers.

The sick person must be at the centre of our preoccupations. Therefore, this alliance between doctors, researchers and patients would seem to us to be of the utmost importance.

To conclude this first part, I would just like to say that the AFM has always had two principal and fundamental objectives which are:

TO CURE and TO HELP. Curing patients suffering from neuro-muscular diseases and at the same time, helping them to obtain compensation which can give them the means to palliate their disabilities.

I now come to the second part of my talk, let’s take a closer look at how this Association functions:
We mustn’t forget that in the AFM, I’m sure you have already understood, it is the patient who is at the heart of all our actions. **It is the patient who has the power and decides the actions.**

The AFM is directed by an executive Board made up of patients and their families. The President has a permanent position, he is the spokesman who guarantees the spirit of the Association. The Board has the executive power. The Association is therefore totally independent and autonomous.

The Board is of course assisted by different experts, financial, scientific as well as medico-social commissions which are extremely important in enlightening the Board before any decision-making: they have an advisory role.

3) All this is possible for the simple reason that we are financially independent thanks to our annual fund-raising event: **the Téléthon**

This event gives us complete autonomy, or at least has always given us up to now complete autonomy.

It has enabled us to change the look in the eyes of the man in the street and shown proof of his natural solidarity and generosity.

This is the perfect opportunity for communicating with the public. Many companies have been our partners for many a year and are entirely committed to giving us their support.

The Téléthon is a wonderful family event which gives us total independence to HELP our families and CURE our diseases.

In a nutshell, I would have you know that:

The financial means from the Téléthon are transformed into genetic information, into understanding the patients and into therapies. Our hope is that maybe one day, we will see our dreams come true and our children will walk.

**CONCLUSION**

To conclude, I would like to remind you that establishing a diagnosis, predicting risks, anticipating certain diseases and treating people are all **responsible actions.**

So, we must constantly

- review our way of thinking
- inform and educate the public
- promote respect for the individual
- be attentive to the wishes of the individual and leave the individual free to decide for himself.
Genetic diseases affect men, women and children of all races, creeds or ages. **We are ALL concerned** and so therefore we must recognize the universal character of these diseases. As a result, we must do everything possible to find a synergy between the individual and the society between what is a right and what is a duty, between solidarity and responsibility.