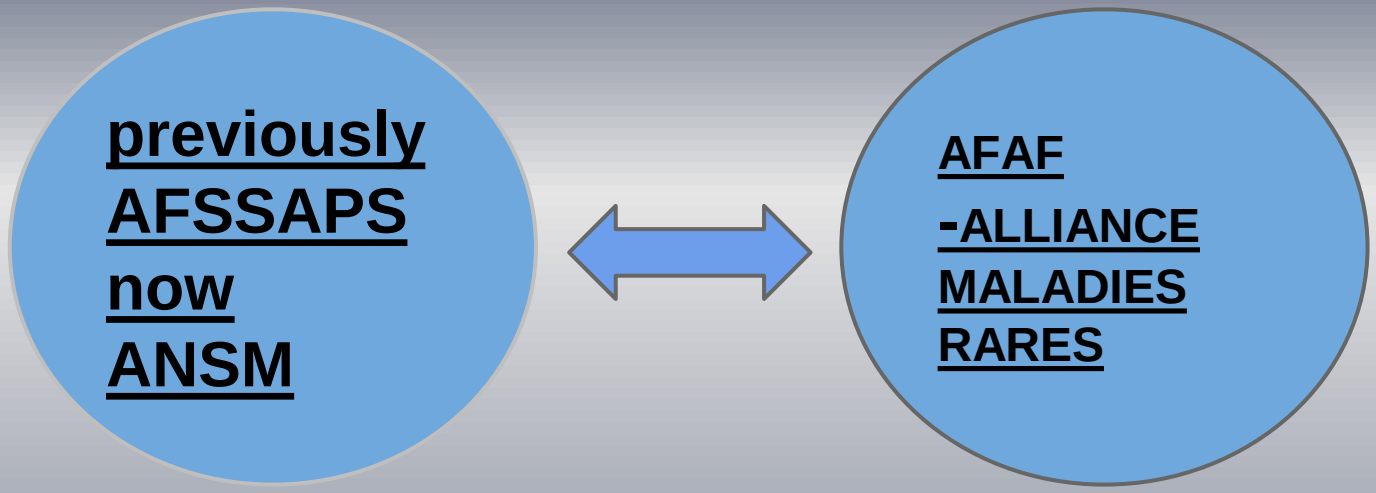
Workshop 2

How to work with your National Competent Authority : a French case

Afssaps and patient organisations



The French Authority **AFSSAPS Agence française.**

Claudie Baleydier : Association Française de l'ataxie de Friedreich et Alliance maladies rares.

How to contact Afssaps and to interact with it ?

An example of interaction between Afssap and Friedreich Ataxia French association

BACKGROUND

Afssaps (now ANSM) :

Point of view of national competent authorities : a very serious crisis because of " mediator scandal "(antidiabetic) used « off label » to loose weight : several deaths so they became very cautious about risk and not about benefits

Point of view of patients: Friereich ataxia is a very serious degenerative disease, shortening life expectancy, no cure, so clinical trials are very important (the previous ones failed)
A.F.A.F : a small association knew very little about Afssaps (few patients representatives)

Background 2

Actos (pioglitazone) an antidiabetic:
because of the Mediator "tsunami" and because of a slight risk of bladder cancer was withdrawn from the market by the French authorities (but not by EMA european medical agency) in July 2011.

A double blind clinical trial using Actos for young ataxian people (20 Actos/20 placebo) was carried on at Hospital Robert Debré in Paris (starting 2010 Ending 2013) funded by government grants and AFAF.

Afssaps threatening to stop the clinical trial using Actos

Afssaps decision to withdraw Actos from the market : June the 9th.

Actos pills have to be brought back to pharmacies on July the 11th.

A letter from Afssap president to the investigator of the trial(Isabelle Husson)on june the 9th : "I intend to stop the trial"

AFAF and others reacting

1) AFAF President 's letter to Afssaps president

2) Letters from "desperate" parents

3) Letters to Afssaps from "big" associations : AFM, Alliance maladies rares supporting Afaf and wanting the clinical trial to continue

4) Several letters from the trial investigator to Afssaps

5) Political reactions(a member of the parliament, the Minister of Health)

6) The pharma companyTakeda reacting

7) European position(a clinical trial has not to be interrupted,pioglitazone is not withdrawn by EMA)

The result :

End of June and July 2011.

The clinical trial may go on with a few changes. Dr Isabelle Husson is tired but happy.

A new problem :
Actos compassionate use
Afssaps having doubt

But now AFAF has better links with AFSSAPS:

- letters fom AFAF president

- letters from parents who were told their children could get Actos till the trial results

- reaction from the pharma companie deciding to give Actos for free

The results : January 2012

Compassionate use of Actos authorised only for the post trial patients since there are no side effects and it is under medical supervision.

General other issues

What about the new rules about off label use so important for rare diseases ?

ATU Temporary use authorisation is changing (more difficult to obtain).

The future and the new law

What about the change from "Afsapp"becoming ANSM?

What about more involvment from patients associations (2 patients representative in ANSM board, 2 patients representatives in every ANSM committee).

What about the future and the new law?

After the mediator "tsunami" French health authorities are Changing.

Afssaps into ANMS : French national authority for medical Security

- How to work with this new institution ?

More involvment of patients associations

- How to work with other French authorities HAS, new Minister of Health

- How to improve our knowledge

Conclusion

- A concrete and specific example from a small association but general problems.

- This was how we worked with the competent authority : it was a kind of struggle but Afssaps gave a better consideration to our case.

Now we know Afssaps organisation better, we know the right people to get in touch with. Afssaps knows that we are a small association with a fighting spirit.

Before the issues we knew nothing about our national authorities, now we know much more.

We dare to react because we knew about clinical trials thanks to Eurordis Summer schools , because we knew bigger associations.

This was a specific case but general knowledge of regulatories is necessary when working with national Authorities.