

French experience with Rare Diseases plans.



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First French National plan on Rare Diseases : 2005-2008

Second plan : 2010-2014

The first plan layed solid foundations for different RD specific activities, mainly by creating centers for expertise.

The second plan aims at building on such foundations the RD world of tomorrow, taking into account the achievements and pitfalls of the first plan, which has been evaluated by different external consultant groups.

**RD patients are rare, as well as RD experts :
expertise is scarce, therefore precious
and has to be shared.....**

**Building,
maintaining
and spreading
expertise is the
result of a
constant
equilibrium.....**



Between

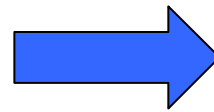
Up-hill

And

Down-hill



Up-hill : beyond the boundaries of the realm of possibilities



need for close connection with :

- fundamental research
- new therapeutic options
- academic and industrial groups involved in orphan drugs.....

Up-Hill

Main achievements of the first national plan on RD 2005-2008 :

- ▶ Support for fundamental research and clinical research including therapeutic trials.
- ▶ Support for genetics labs (up-grading)
- ▶ Support for animal models

Main projects for the second plan :

- ▶ Create a foundation for scientific cooperation in charge of :

Advising for scientific projects and grant submissions

Help for access towards highly specialised tools

Promoting partnerships between academic and industrial stake holders for

Down-hill :



**From centers of expertise
to patient home**

**Spreading information,
for management and therapeutic
guide-lines is time consuming,
and involves several levels of
stake-holders.**

Spreading expertise

First plan, main achievements :

- ▶ **131 centers of expertise**
- ▶ **500 « Competence » centers**
- ▶ **Orphanet**
- ▶ **Better connection between patient organisations and experts.**

Second Plan, main projects :

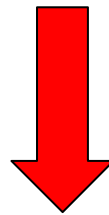
- ▶ **National networks for diffusion of expertise, information, and therapeutic education.**
- ▶ **Local assistance for home care and social problems : experimental systems in different regions.**



**Expertise is
bicephalous,
involving :**

**▶ In the one hand
clinical management
and follow-up.**

**▶ In the other hand biological assessments by
highly specialized and constantly evolving
approaches.**



**Officially recognized partnership in
the second plan.**

CONCLUSION

**Maintaining equilibrium
is a constant challenge
And...**



**No aim can be achieved
without a network**