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Rare Diseases Organisation

Development of a National Plan Rare Diseases

The **Belgian** experience



Some history (1)

Pioneers:

Lut De Baere (patient organisation Boks)

Yolande Avontroodt (MP)

Erik Tambuyzer (pharma)

Jean-Jacques Cassiman (genetician)



Some history (2)

- Informal meetings
- Attract more stakeholders
- Voice of the patients: patient umbrella organisations VPP, LUSS, PR&T

RaDiOrg.be didn't exist yet !



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Let's get started!

RaDiOrg.be is born in 2008

- 1st Rare Disease Day 2008
in federal parliament
patronage of princess Astrid
- + all stakeholders => Fund Rare Diseases & Orphan Drugs



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Let's get started!

- In the framework of the '**Programme for the amelioration of the life quality of patients with a chronic disease**', the Fund Rare Diseases and Orphan Drugs has been supported by the Minister of Social Affairs and PublicHealth, Ms. Laurette Onkelinx.
- The Fund, managed by the King Baudouin Foundation, got the specific assignment, through a contract with the Belgian National Institute for Health and Disability Insurance (RIZIV/INAMI), to delineate in close collaboration with the stakeholders **recommendations and proposals for measures which could form the basis of a Belgian Plan for Rare Diseases.**



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Multistakeholders group





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7 Parallel working groups

1. National registry, databases and European collaboration
2. Identifying hidden non-medical cost for patients/towards a road map
3. Information for patients, health professionals and the public – Patient empowerment
4. Centres of competence, centres of expertise and national collaboration
5. Access to and financing of diagnosis, medication, treatments and patients management
6. Fundamental research and clinical trials
7. Training and education of health professionals



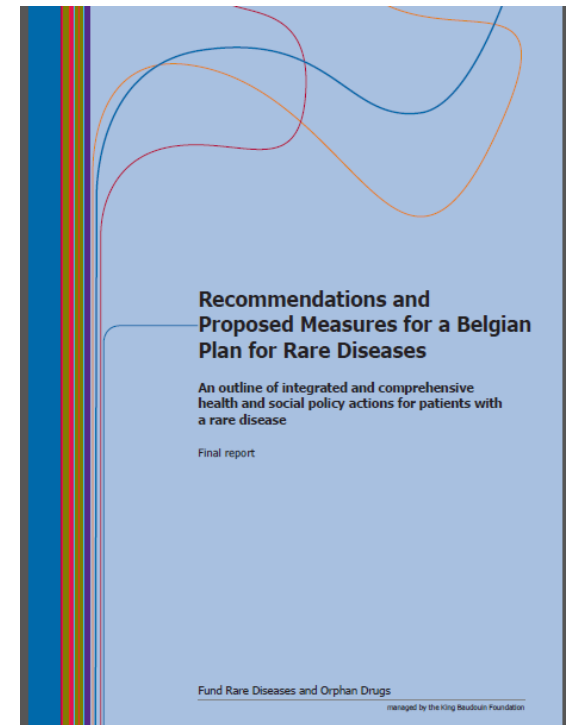
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Patients representation

- At least 1 in each working group
- Mainly Radiorg board members (4 to 5; in the beginning only 1) + VPP representative
- Patient rep. chaired 2 important working groups: n°2. identifying non-hidden costs and n°3.information & patient empowerment

Results

1. Phase 1 : interim report handed over to the Minister in May 2010.
2. Final report handed to the Minister in October 2011.



The “plan” in a nutshell

The 42 recommendations and proposed measures in this report cover 5 main principles:

- expertise and multidisciplinary
- collaboration and networking
- knowledge, information and awareness
- equity in access
- governance and sustainability



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Where do we stand today?

- 2 years of hard but constructive work
- Minister didn't approve this "plan" as a whole but

=> Budget for implementation of 5 key measures.



Measures already implemented

1. New “nomenclature” for genetic tests (1.1.2013)
2. Reimbursement of DNA-analysis outside Belgium (1.1.2013)

Measures in implementation phase

- RIZIV/INAMI & FOD Health: priority measures
 - no more patient involvement in this phase
- In process (WG with patient rep. + mission for ISP)
 1. national register
 2. inventory of centres of expertise



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More measures

- Orphanet Belgium (NL + FR)
- New mechanism for early temporary access and reimbursement

Difficulties from PO's perspective (1)

- Lack of expertise in communicating real life experience
- Lack of time due to illness or other professional commitments
- Lack of respect by other stakeholders
- Lack of coordination & coherence
- Lack of funding for supporting patients' involvement

Difficulties from PO's perspective (2)

- No part in final implementation phase
- Only official PO representation is in Observatory Chronic Diseases (Radiorg via VPP and LUSS)
=> recently: permanent WG in Observatory for Rare Diseases (after pressure from Radiorg and the Fund stakeholders)





Thanks!

Further info:

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