

# **Experiences in The Netherlands: Networking with patients, professionals and industry**

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***Dutch Steering Committee Orphan Drugs***

## **European Regulation Orphan medicinal products (Nr. 141/2000, 16/12/1999)**

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- **Stimulating measures of European Committee**  
(market exclusivity of 10 years, central registration in EU, scientific advice, reduction of costs)
- **Stimulating measures of individual member states**  
**As an example: *The Netherlands***

## Policy developments in The Netherlands (Ministry of Health, Welfare and Sports)

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- **1998:** Recommendations of the Dutch Advisory Council on Health Research (RGO)
- **2001:** Appointment of the *Steering Committee Orphan Drugs* by the Minister of Health, Welfare and Sport (VWS) in April 2001
- **2004:** Evaluation of the *Steering Committee Orphan Drugs*

## Dutch Advisory Council on Health Research

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- **Consultations (1997-98) with:**
  - Patients**
  - Physicians/researchers**
  - Pharmaceutical industry**
- **Analysis of five case studies**
- **Committee Orphan Drugs that prepared this advice consisted of several stakeholders**

## Advice of this council (1998): main points

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- Make an inventory of and co-ordinate ongoing initiatives, stimulate new initiatives
  - ▶ **via a national organisation with an independent steering committee**
  
- Prioritise on a European level 25-50 rare disorders
  - ▶ **stimulate industry to develop medicines in these areas**
  
- Stimulate research and development
  - ▶ **create financial support, tax agreements, feewaivers, a reimbursement policy**

## Government policy

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- **Steering committee OD** → established in 2001
- **Prioritising certain rare disorders** → dismissed (for the time being)
- **Stimulating measures** → working on some proposals (e.g. developing a reimbursement policy)

## **Steering Committee Orphan Drugs: Dutch network between stakeholders**

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### Collaboration with stakeholders

- **Necessary for successful research, treatment and care for patients with a rare disorder**
  
- **Each stakeholder has**
  - **joint interests**
  - **own interests**

## ***Steering Committee - composition***

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- **Ten members on personal title from:**
  - **Two umbrella organisations of patients' support groups**
  - **Two medical university professors**
  - **University pharmacist (childrens hospital)**
  - **Two umbrella organisations of pharmaceutical industry**
  - **Medicines Evaluation Board**
  - **Health Care Insurance Board**
  - **Chair – university professor pharmacoepidemiology**
  
- **Observer from:**
  - **Ministry of Health, Welfare and Sports (also member of the COMP)**



## Mission of the *Steering Committee*

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The *Steering Committee* on Orphan Drugs has the following mission:

- Stimulate the development of orphan drugs
- Improve the situation of patients with a rare disease, especially strengthen the transfer of information on rare diseases

## Funding and time-horizon

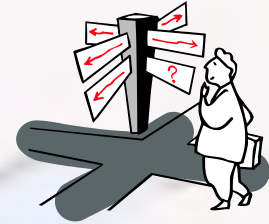
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- **The *Steering Committee* was installed for four years (2001-2004), followed by an evaluation. Recommendation of RGO: install for at least 10 years**
- **The results have been evaluated in 2004. The Minister has decided to continue the subsidy for this committee up to 2008**
- **The ministry of Health has made available an annual budget of 450,000 Euro in 2001-2004. Similar budget for the next three years**
- **The secretariat of the committee is situated at the Netherlands Organisation for Health Research and Development (ZonMw) – two scientific officers**

## Target groups of the *Steering Committee*

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- **Patients with a rare disease**
- **Physicians, pharmacists**
- **Researchers (fundamental and applied research)**
- **Pharmaceutical industry**
- **Health insurance companies**
- **Administrative bodies**
- **Politicians**
  
- **and their associations**



## Methods of the *Steering Committee*

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- **Bottom-up approach: discussions with target groups**
- **Invitational Conference: discussion of identified issues with target groups (November 2001)**
- **Dutch Symposium: introduction of activities to public (January 2002)**
- **Website [www.orphandrugs.nl](http://www.orphandrugs.nl) (April 2002)**
- **Working groups (started in 2003)**
- **Orphan Café (in 2004)**

## Working groups

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### **Composition**

Several members of *steering committee* and 6 members of different target groups

### **Aim**

To analyse several key issues in depth and to report to the *steering committee* on the bottlenecks and solutions.

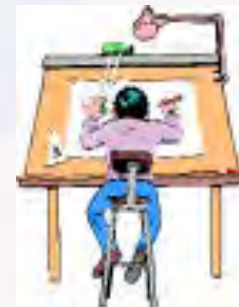
### **Themes**

- Website - information
- Education
- Research
- Epidemiology
- Availability of orphan drugs

## Steering Committee Orphan Drugs

### Functions

- Information desk
- Booster
- Architect
- Brainpower



**Collecting information on rare diseases in The Netherlands and making it available for the target groups**

***Results***

- **Dutch website ([www.orphandrugs.nl](http://www.orphandrugs.nl); 30 April 2002)**
- **Working group Website: building connections with other websites**
- **Answering questions (by telephone and e-mail)**
- **Articles, brochures, newsletters, presentations**
- **Conferences**
- **Working group Education: information for physicians, pharmacists and students from universities**

**Facilitating and encouraging fundamental and clinical research and industrial activities concerning rare diseases in The Netherlands. Connecting with international initiatives.**

### ***Results***

- **Grant to develop a national programme Rare diseases and Orphan drugs and to stimulate industrial activities (via an orphan developer) (2005-2008)**
- **Working group Research: inventory of ongoing Dutch research**
- **Working group Epidemiology: inventory of protocols for registration of rare diseases**
- **Participation in international projects (E-Rare, Priority Medicines, Orphan Platforms, ERDITI, EPPOSI Workshops)**



International projects on research on rare diseases

**Priority Medicines** to prepare a public-health-based medicines development agenda

**E-Rare** comparison on level of research programmes

**Orphan Platform** to develop information tools to exchange information on research on rare diseases and its coordination

**ERDITI** European Rare Disease Therapeutic Initiative

Network of stakeholders

**EPPOSI Workshops** European Platform for Patients Organisations, Science and Industry

**Funding of projects and analysing procedures to improve access of orphan drugs**

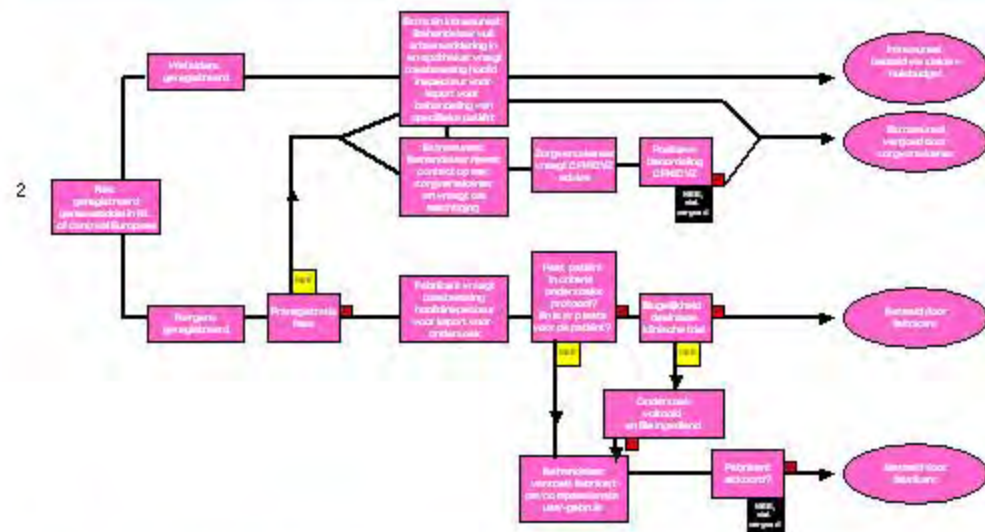
### ***Results***

- **Project: Dutch umbrella working group of patients with a rare disease (2002-2004)**
- **Project: Bottlenecks in care for patients with a rare chronic disease' (including an English bibliography) (2003)**
- **Funding a project on diagnosis of mitochondrial diseases (2004)**
- **Working group Availability of orphan drugs: Analysis of access of authorised and non-authorised drugs for patients**

Registered in NL or in EU



Not registered in NL or in EU



Pharmacy preparation



**Stimulating a debate about orphan drugs in order to improve the 'climate' for diagnostics and treatment of rare diseases**

### ***Results***

- **Orphan Cafés: Debating a specific issue with two guest speakers and with the several target groups in an informal way – creating networks**
- **Participating in ongoing debate on orphan drugs**
- **Signalling dilemmas concerning orphan drugs and giving invited and uninvited advice (e.g. on reimbursement issues and access issues)**
- **Informing members of parliament**

## **Issue on Dutch reimbursement policy (2000-2005)**

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### **How to reimburse orphan drugs?**

- **Orphan drugs are reimbursed:**
  - **outpatients: paid by the regular reimbursement system for pharmaceutical products**
  - **inpatients: paid by the hospital**
  
- **Exceptions in cases where the added therapeutic value is not (yet) evident ▶ additional studies will be required:**
  - **outpatients: paid by reimbursement via state support system (€ 34 million/year)**
  - **inpatients: paid by the hospital**

## Issue on Dutch reimbursement policy -2

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- **Discussion about costs of treating patients in (academic) hospitals with orphan drugs between House of Representatives of Dutch Parliament and the Minister of Health**
- **Close collaboration between patients, physicians, industry and boards/associations of (academic) hospitals in this discussion**

## What is the Dutch *Steering Committee* on Orphan Drugs adding to the picture ?

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- **Network of stakeholders**
- **Developing ‘interfaces’ (informal networks, orphan café, etc.) between scientists, industry, patient groups, clinicians, regulatory affairs, etc.**
- **Collecting and giving information**
- **The establishment of the position of an ‘orphan developer’ and the development of a national funding programme for rare diseases**
- **Ongoing ‘political pressure’ on topics of reimbursement of orphan drugs and pharma innovation**
- **EU collaboration**

## Evaluation by the Ministry of Health 2001-2004

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- **Total impression very positive**
- **Availability of information for patients and other stakeholders improved**
- **More attention to rare diseases and orphan drugs in The Netherlands**
- **Network of relevant persons, organisations and institutes is present now**
- **Continuation of subsidy for three years (2005-2007)**



## Evaluation by target groups: 2001-2004

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- Representatives of the target groups are in general positive about the (activities of the) *steering committee*
- The different target groups have many expectations that are not always realistic with the present knowledge on rare diseases and with the current budget and personnel of the *steering committee*
- International collaboration is very important

## Ministry of Health: recommendations for next years

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### ➤ Information

- Communication plan
- Specific groups

### ➤ Research

- Follow, coordinate and encourage research and attention for care for rare diseases in academic hospitals
- High priority on research and development of medicines

### ➤ Involvement of the *steering committee* in consultations on reimbursement

## Experience of the *Steering Committee* (1)

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### *Evaluation of the steering committee itself*

- **Positive about the multidisciplinaryity of the *steering committee* and working groups: looking across the boundaries of his/her own discipline**
- **The existing members want to stay in the *steering committee***
- **A representative of the health care insurance companies will join**

## Experience of the *Steering Committee* (2)

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- **A broad mission**
- **Complex issue that needs time**
- **Economic recession in The Netherlands influences the 'orphan' climate**
- **International collaboration very important**

## Experience of the *Steering Committee* (3)

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- **First years were mainly used for giving information, inventory activities and networking**
  
- **In the forthcoming years important issues:**
  - **Communication/PR**
  - **Monitoring – building models**
  - **Research and innovation**
  - **Securing dossier from 2008 onwards**
  
- **Would appreciate similar steering committees in other European countries**



## ***Steering Committee Orphan Drugs***

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