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ICORD 2010

Orphan drug regulation and national strategies: complementary actions

Sonja van Weely

Dutch *Steering Committee* on Orphan Drugs

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Regulation (EC) 141/2000 for Orphan medicinal products

- Incentives for development and marketing of orphan medicinal products for the European Union, meant for diagnosis, prevention or treatment for rare conditions
- However, availability and access for patients is different in the EU member states
- Multiple reasons, e.g.
  - National procedures for reimbursement
  - Companies do not market their product in small countries as the first step
- Other actions are needed to improve the possibilities for reimbursement for orphan drugs

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National actions/plans/strategies are needed

- **Especially needed for patients with rare disorders for whom no therapy is available**
- **Building blocks of the Council Recommendation (2009/C 151/02) on an action in the field of rare diseases:**
  - **Definition, codification and inventory**
  - **Research**
  - **Centres of expertise and European reference networks**
  - **Gathering expertise at European level**
  - **Empowerment of patient organisations**
  - **Sustainability of infrastructures in the field of information, research and healthcare for rare diseases**

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Parallel developments in Europe and in The Netherlands

European Community:

- Consultations with stakeholders on possible legislative procedures (starting in 1995)

▼

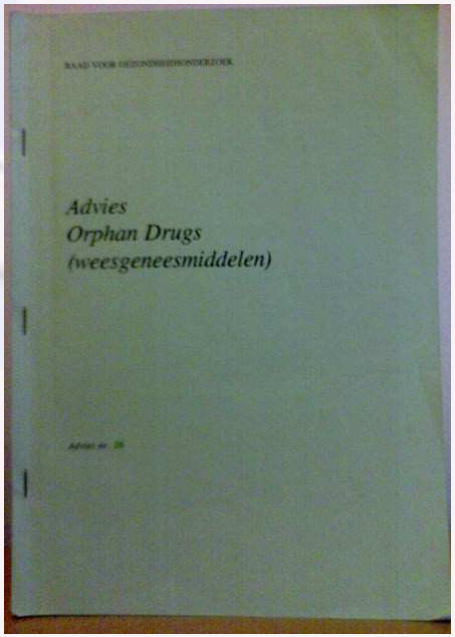
- Regulation (EC) 141/2000 on orphan medicinal products and Rare disorders

▼

- Committee for Orphan Medicinal Products at the European Medicines Agency

The Netherlands

- Consultations with stake-holders starting in 1998:



Advice from Dutch Advisory Council on Health research (RGO)

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Main recommendations of the RGO and resulting government policy

- Make an inventory of and co-ordinate ongoing initiatives, stimulate new initiatives ► via a national organisation
- Stimulate research and development ► create financial support, tax agreements, fee waivers, a reimbursement policy

- *Steering committee* established in 2001
- Some proposals accepted or in preparation, some dismissed

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Dutch *Steering Committee* on Orphan Drugs

- Installed in 2001 by Dutch Minister of Health, Welfare and Sport
- Financed by this ministry
- Independent

- Eleven members on personal title from:
  - Two umbrella organisations of patients' support groups
  - Two medical university professors
  - University pharmacist (Children's hospital)
  - Two umbrella organisations of pharmaceutical industry
  - Dutch Medicines Evaluation Board (MEB)
  - Health Care Insurance Board (CVZ)
  - Health Insurance Company (since 2005)
  - Chair – clinical pharmacologist/former chair of MEB

- Observers from:
  - Ministry of Health, Welfare and Sport
  - Dutch member of the COMP/EMA

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Mission of the *Steering Committee*

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

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

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Strategies on availability and access of orphan drugs

- Research
- Reimbursement
- Information

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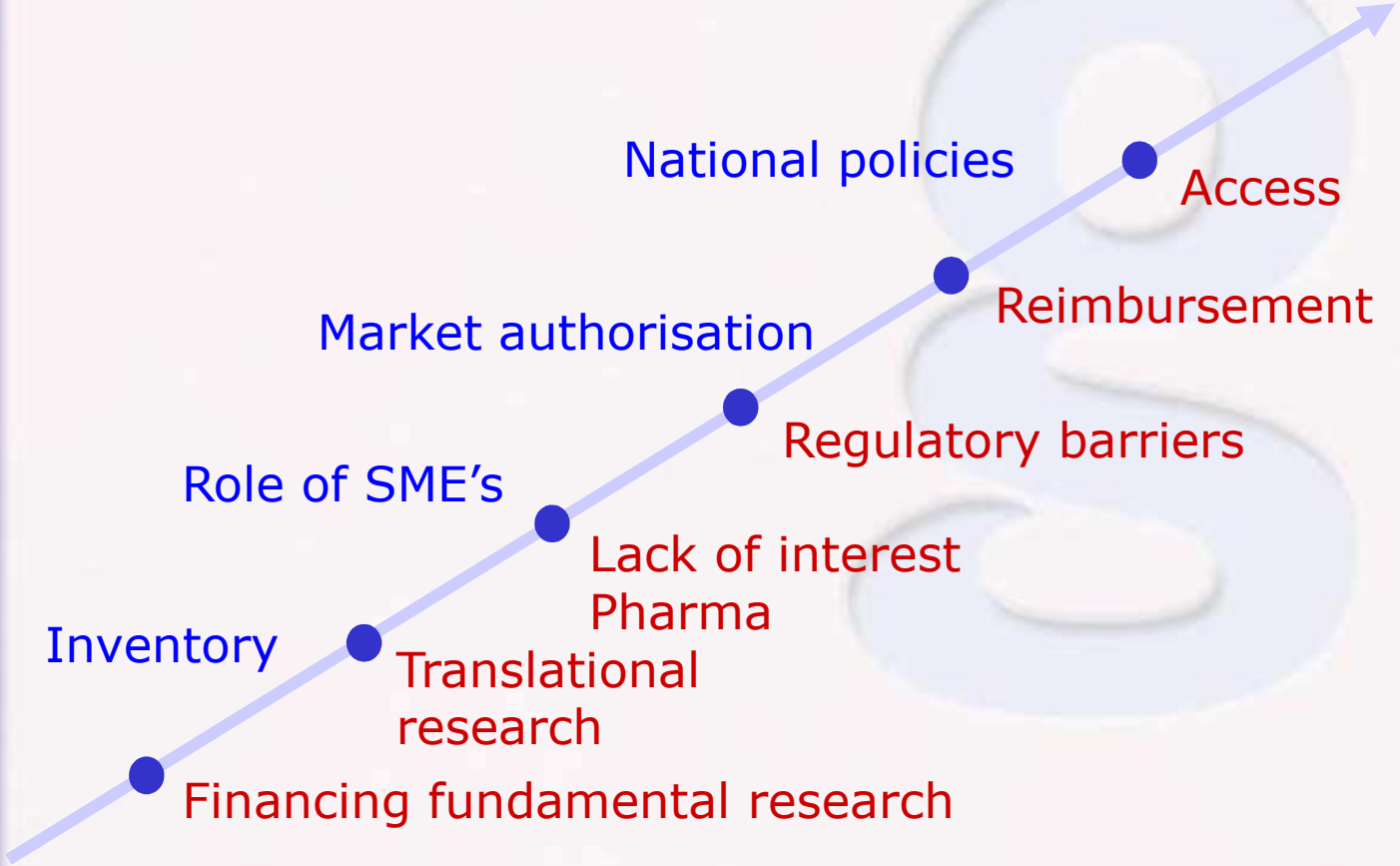
Key issues in Orphan Drug development



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Research and Technology

Four year programme with total budget of EUR 0.5 million

Aim:

- **To encourage the development of orphan medicinal products in The Netherlands and elsewhere**

- **Two projects:**

1. **‘Orphan product developer´: acts as mediator between Dutch universities, university medical centers, technology transfer points, SME´s and other pharmaceutical companies**

2. **PhD student – research on rare diseases and orphan drugs**

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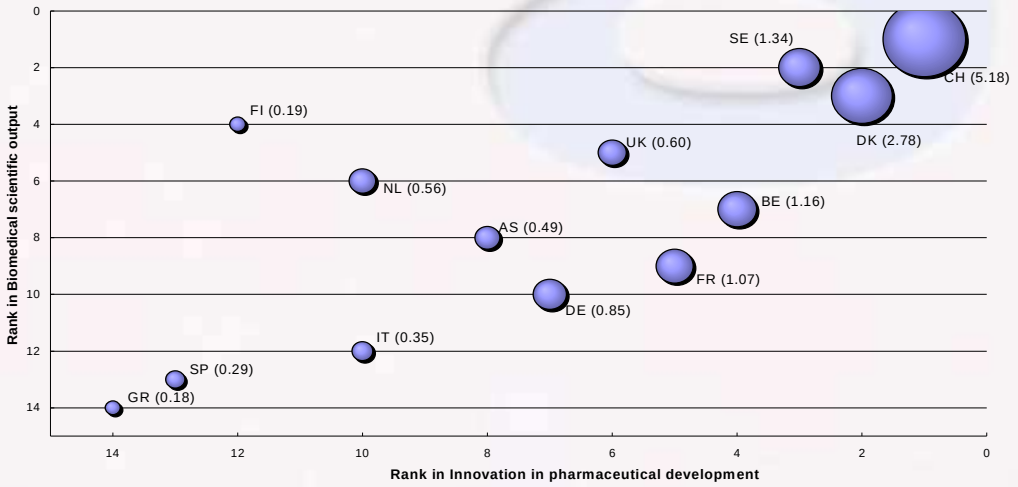
Project 2: a PhD-project (1)

- **Main deliverable: thesis**
- **Identification of success factors for enhancing the development of orphan drugs: for example**
  - **basic biomedical research**
  - **strong pharmaceutical innovation in general, including patent applications, R&D expenditure and the existence of small and medium enterprises ¨**



Heemstra HE et al. Drug Discov. Today 2008; 13 (15-16): 670-676

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Project 2: a PhD-project (2)

- **Second main deliverable:**
  The Development of a Proposal for a Scientific Programme:

- **To encourage precompetitive translational research with the ultimate goal to develop treatments (key words: focus and mass, involvement of patients, perspective on participation of industry)**

- **To encourage international collaboration and participation in E-Rare (European project on rare disease research)**

- **The programme will start in 2011; 13.6M€ available**

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Strategies on availability and access of orphan drugs

- Research
- **Reimbursement**
- Information

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Definition of a rare disease used for reimbursement

- A life-threatening or chronically debilitating condition affecting not more than 5 in 10,000 persons in the European Community (EC)
- Exception: In case of off-label use of registered medicinal products for patients with a prevalence of less than 1:150,000 inhabitants in The Netherlands: reimbursement despite the fact that the product is not registered for the specific rare indication

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Dutch assessment for reimbursement of (orphan) drugs

- In principle similar procedure of assessment for orphan and non-orphan drugs by a specific committee of the Health Care Insurance Board
- Contents of extramural reimbursement dossier:
  - Pharmacotherapeutic evidence (therapeutic added value):
  - Pharmaco-economic evaluation (dispensation for orphan drugs!)
  - Budget impact

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Reimbursement of medicines (1)

- **Medicines in Primary Care and Out-patient Setting:**

1. **Average of prices in UK, Germany, Belgium, France**
2. **Health Care Insurance Board advises on reimbursement, but the government decides ►**
3. **Medicines are put on one of the following Lists:**

- **List 1A** — **Medicines put into groups; with upper limit of reimbursement**
- **List 1B** — **Unique medicines, fully reimbursed, no upper limit of reimbursement**
- **List 2** — **Limitative preconditions for reimbursement**

- **Unique orphan drugs are on List 1B**

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Reimbursement of medicines (2)

- **In-Hospital treatment:**
- **Diagnosis/treatment combinations (DBCs)**
- **In case there is no DBC and the prescriber in the hospital decides to prescribe the medicine, the hospital has to pay for it by the hospital budget**
- **Policy Rules for Expensive Medicines and for Orphan Drugs**

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Policy rules (1)

- Only for In-Hospital treatment
- Conditional reimbursement for four years
- Additional evidence on (cost)-effectiveness has to be obtained in these four years
- New research programme for collecting more data on (cost-) effectiveness
- Start of the first projects: 1-10-2008

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Policy rules (2)

- **In the case of policy rule for orphan drugs:**
- **European Orphan Drug Designation Status mandatory**
- **Only in University Hospitals - to improve expertise building**
- **Threshold for application for policy rule for orphan drugs: Expenditure on an orphan drug exceeds 600,000 € in the individual university hospital**

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Policy rules: reappraisal after four years

- **Health Care Insurance Board reappraises the available evidence on efficiency in daily practice upon four years:**
  - **Therapeutic value**
  - **Actual costs of the medicinal product**
  - **Cost-effectiveness**
  - **Efficient prescription**

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Strategies on availability and access of orphan drugs

- Research
- Reimbursement
- **Information**

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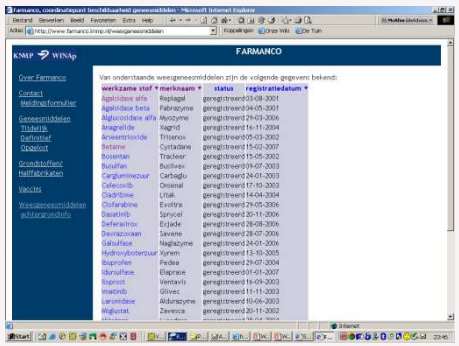
Information on availability of (orphan) drugs

- **Brochure for clinicians and pharmacists**

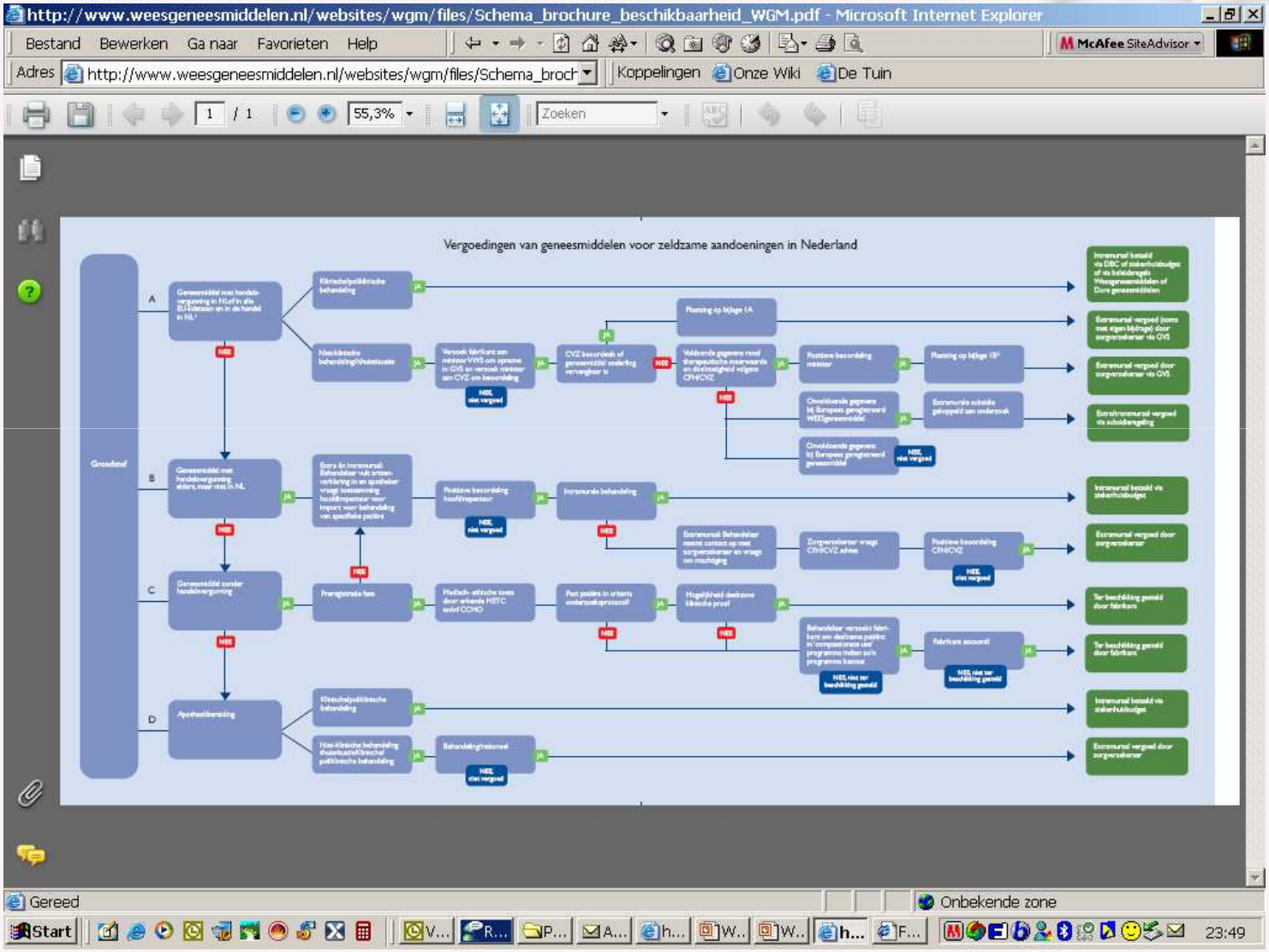
  and

- **Brochure for patients**

- **In collaboration with the Dutch Royal Society of Pharmacists: website with information on aspects of European registered orphan medicinal products (www.farmanco.knmp.nl/ weesgeneesmiddelen)**



Situation → **Essential actions to be done by practitioner and pharmacist** → **Way of reimbursement**



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Conclusions

- Orphan Drug Regulations and national plans/strategies should complement each other
- The Netherlands has chosen to develop strategies involving:
  - Multidisciplinary approach
  - Inventory of existing situation
  - Performing research to come up with solutions and to monitor the solutions
  - Disseminate information to several stakeholders

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Muchas Gracias!

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