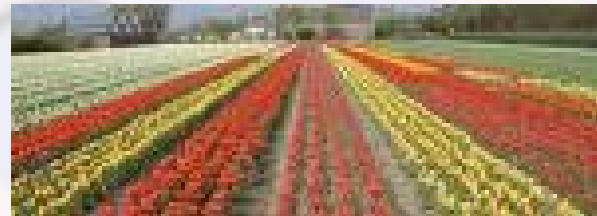


# Policies on rare diseases research and orphan drugs in The Netherlands



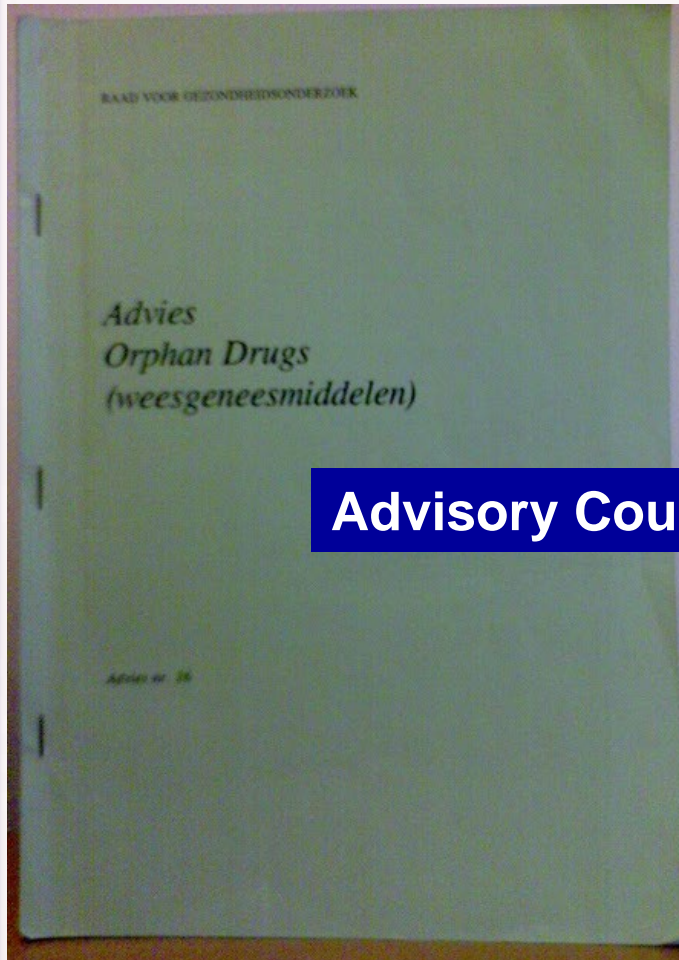
**Sonja van Weely**

**Dutch *Steering* Committee on  
Orphan Drugs**

## 1998: Start policy on rare diseases in the Netherlands



**Minister of  
Health, Welfare  
and Sport**



**Advisory Council on Health Research (RGO)**

### **Background:**

EU Policy on rare diseases in the Making: Regulation (EC) 141/2000

## **Dutch *Steering* Committee on Orphan Drugs**

---

- **Installed in 2001 by the Dutch Minister of Health, Welfare and Sport (VWS)**
- **Financed by this ministry**
- **Independent organisation**
- **Multidisciplinary composition:**  
physicians, researchers, representatives of patients organisations, pharmaceutical industry, governmental bodies (Dutch Medicines Evaluation Board, Health Care Insurance Board ), Health Insurance Company (since 2005)
- **Chair: Hubert Leufkens – university professor pharmacoepidemiology**
- **Observers from:**
  - **Ministry of VWS**
  - **Dutch member of the COMP/EMEA**

## **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**

**Encouragement of research on rare diseases and orphan drugs has been a major issue from the start of its activities**

## **Funding of (medical) research in The Netherlands**

---

- **Direct government funding to the eight universities/ university medical centres**
- **Indirect government funding via The Netherlands Organisation for health research and development (ZonMw)**
- **Charities, pharmaceutical companies, patients organisations, etc.**

**In general similar for (medical) research on prevalent and on rare diseases**

## **Analysis of ZonMw programmes on rare diseases research (2002-2006)**

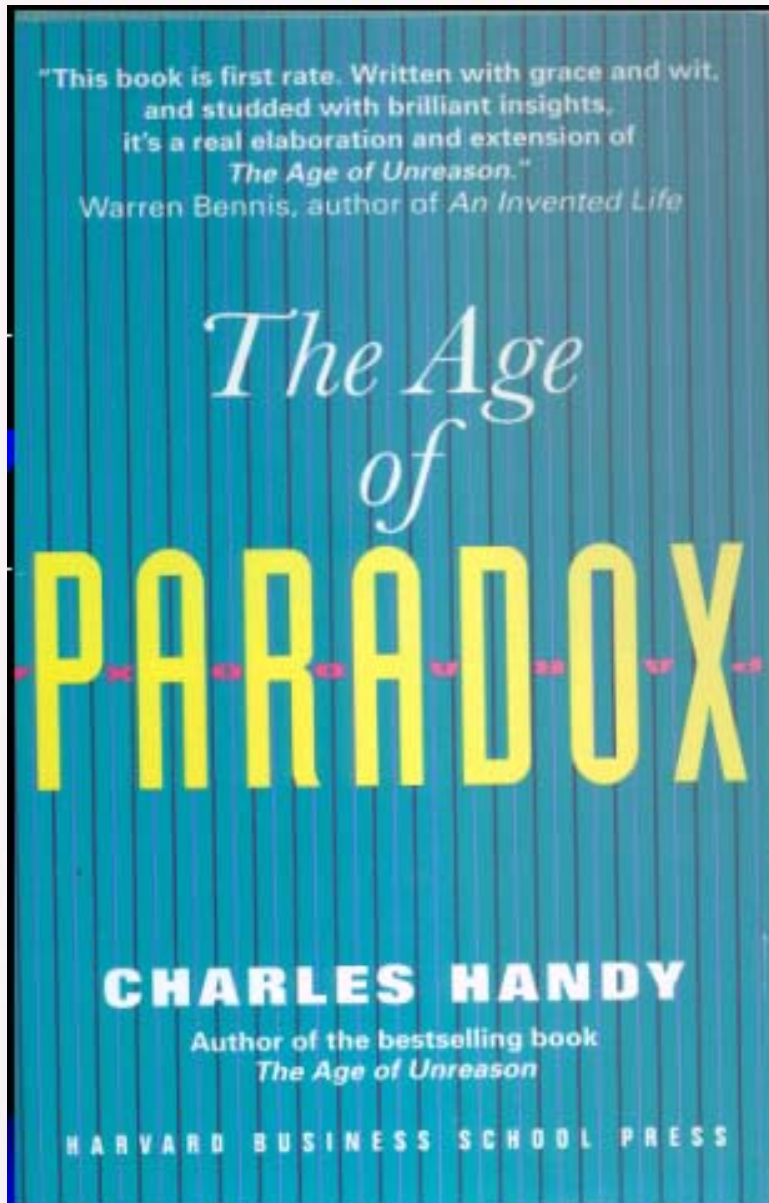
**A**

- **A collaboration of Harald Heemstra, PhD student at the University of Utrecht, ZonMw and Dutch Steering Committee**
- **Systematic inventory of the internal database of ZonMw of about 85 programmes for the period 2002- 2006**
- **Projects were classified as 'rare' if the prevalence of the main disease of interest was less than 5/10,000 (European definition)**
- **Information collected on ZonMw programme, granting, disease category, research area, university and budgets**

## Analysis of ZonMw programmes: conclusion

---

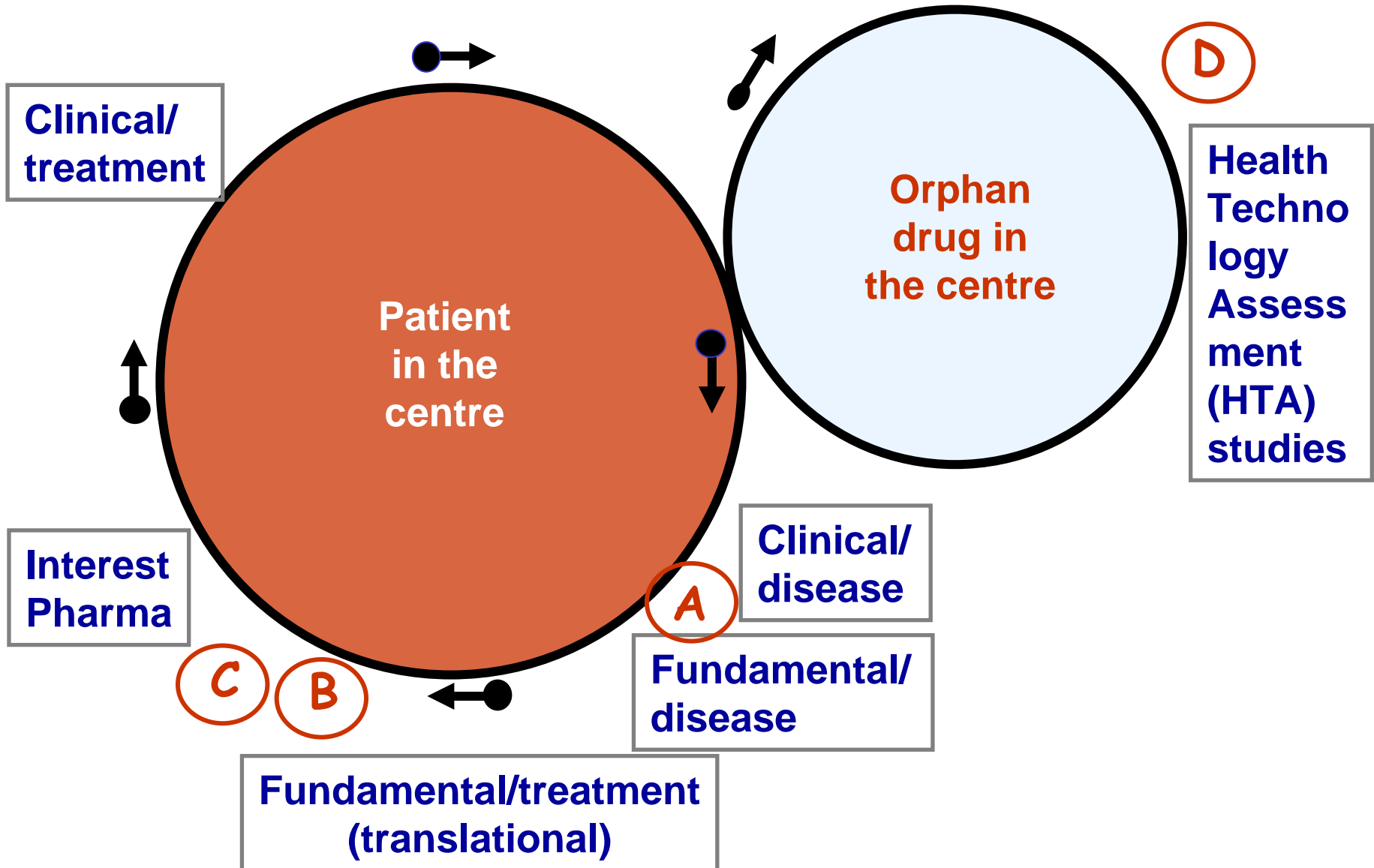
- Rare disease research *is* funded by ZonMw without specific additional incentives:
  - On average similar granting percentages as for other projects
- Rare disease research is spread irregularly over the programmes
- Relatively more funding towards fundamental research (e.g. pathophysiology / animal models)
  - Higher number of applications
  - Higher percentage grants awarded
- Most applications for oncology and musculoskeletal/ nervous related research



**The Dutch paradox:**  
**Strong in**  
**fundamental science**  
—  
**poor in (translational)**  
**innovation**



# Initiatives in rare disease research from bench to bedside to bench to ...



## From fundamental research to orphan products (1)

---

- A** Keep the existing bottom-up approach programmes to apply for 'rare disease' projects: the fundamental research will be continued
  
- B** New ZonMw programme proposal (5y) entitled 'Rare diseases and orphan drugs: from orphanised to cured' (submitted to ministry of Health in April 2007)
  - Aim 1: Encourage precompetitive translational research with the ultimate goal to develop treatments (key words: focus and mass, past performance, involvement of patients, perspective on participation of industry)**
  - Aim 2: Encourage international collaboration e.g. join E-Rare calls**

## From fundamental research to orphan products (2)

---

### **C** Programme STIGON-Weesgeneesmiddelen (2005-2010)

**Aim: Encourage development of orphan medicinal products**

**Two projects:**

- **Analysis of research on rare diseases and orphan drugs - PhD student H. Heemstra**
- **‘Orphan product developer’ R. de Vruh - acts as mediator between Dutch universities, university medical centers, technology transfer points, SME’s and other pharma companies and informs them about Orphan Drug Regulation, etc.**

## Research on added value of an (orphan) product

---



### Programme Expensive and orphan medicines 2007-2014

**Aim: Investigate the effectiveness of expensive and of expensive orphan medicinal products**

**to help the Dutch Health Care Insurance Board in its advice on further *reimbursement* of products that are temporarily admitted for three years to the lists of the policy rules of the expensive or orphan medicinal products**

## Conclusion

---

- **Fundamental research on rare diseases is funded in existing programmes of ZonMw**
- **New programmes or initiatives have been developed based on the concept 'from bedside to bench to bedside'**
- **Encouragement of translational research to development of therapies**
- **Encouragement of multidisciplinary collaboration and of international collaboration in rare diseases research**

**wgm**

STUURGROEP WEESGENEESMIDDELEN



**Thank you for your attention**

**Steering Committee Orphan Drugs**

**PO Box 93245**

**2509 AE The Hague**

**The Netherlands**

**wgm@zonmw.nl**

**www.orphandrugs.nl**