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Policies on rare diseases research and orphan drugs in The Netherlands

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Dutch *Steering* Committee on Orphan Drugs

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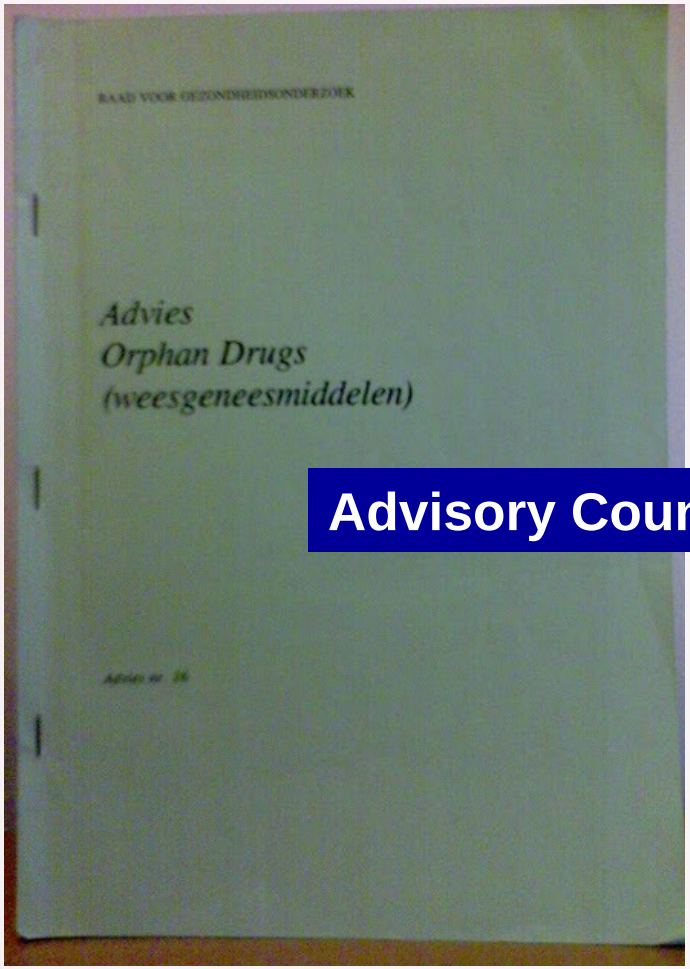
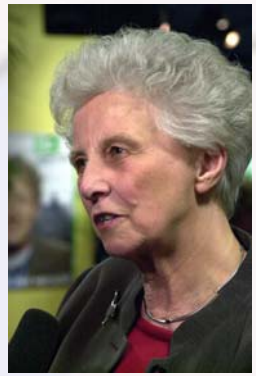
1998: Start policy on rare diseases in the Netherlands





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

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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**

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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**

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

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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

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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

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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

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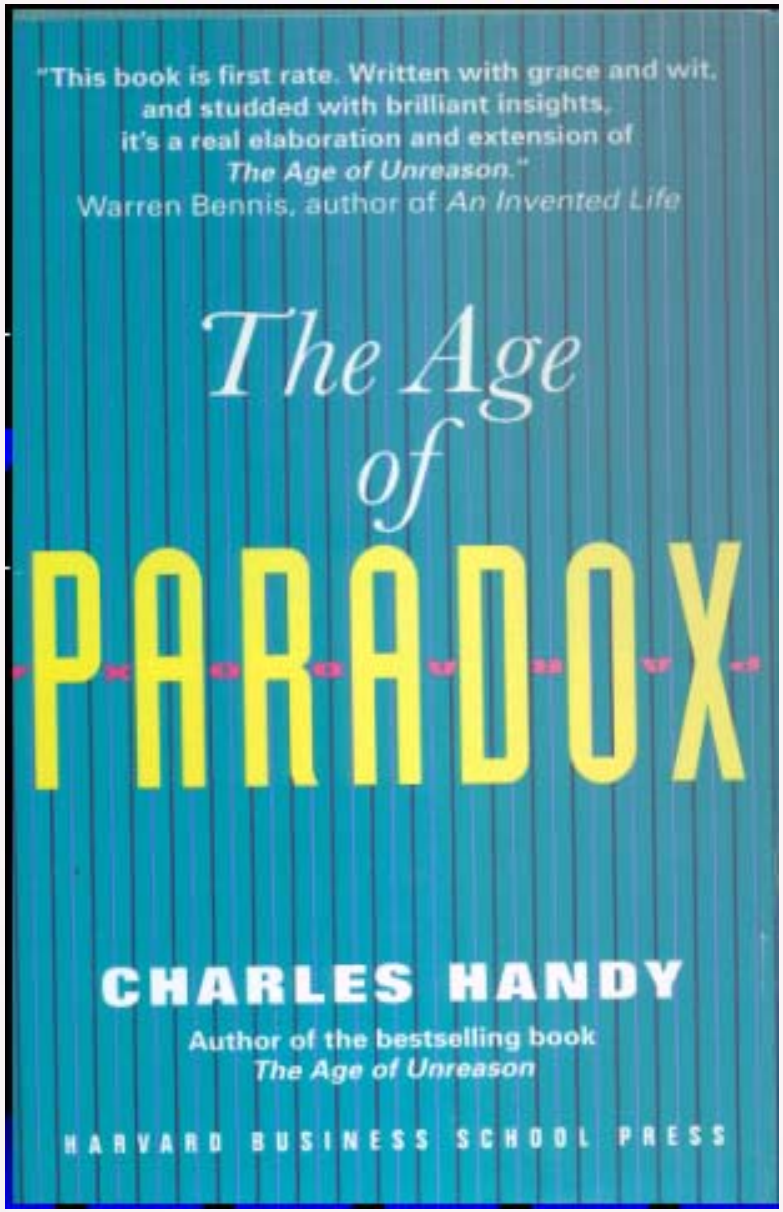

The Dutch paradox:

Strong in fundamental science

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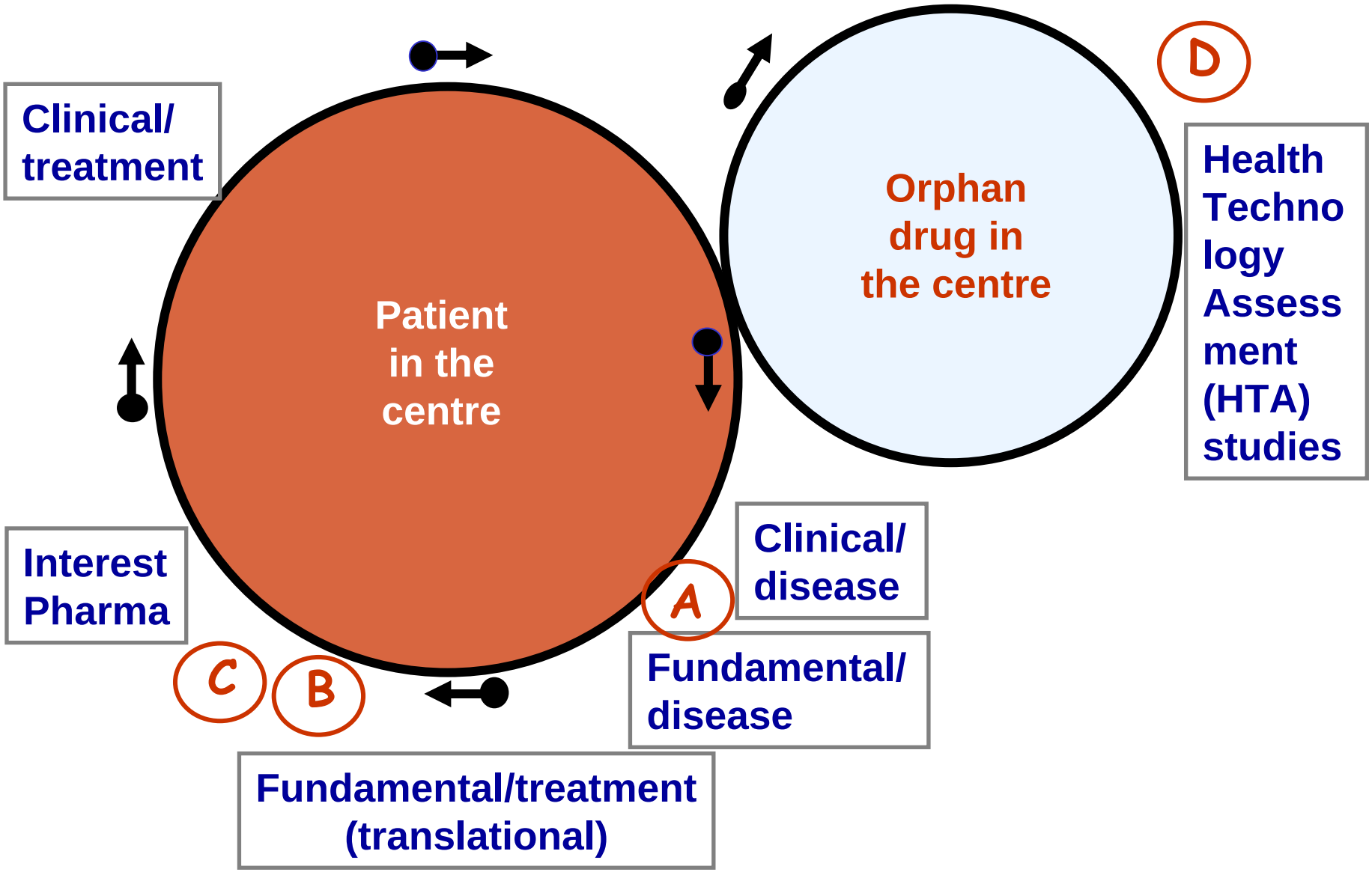
poor in (translational) innovation

Initiatives in rare disease research from bench to bedside to bench to …



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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

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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 Vrueh - 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.**

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Research on added value of an (orphan) product

D

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

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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

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Thank you for your attention

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