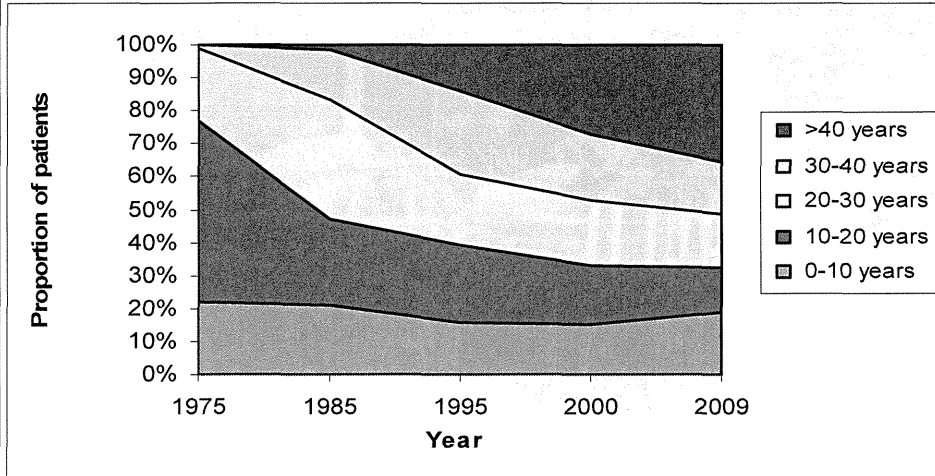


Age-distribution severe haemophilia (n=338)



Crevelddclinic, Utrecht, 2010, personal communication

Aging

with

haemophilia

Medical
and
psychosocial
impact

E.P. MAUSER-BUNSCHOTEN
A. DE KNECHT-VAN EEKELÉN
C. SMIT

Table 1. Persons with Hemophilia Who Developed HIV Infection from Transfusion of Contaminated Blood Products in Selected Countries*

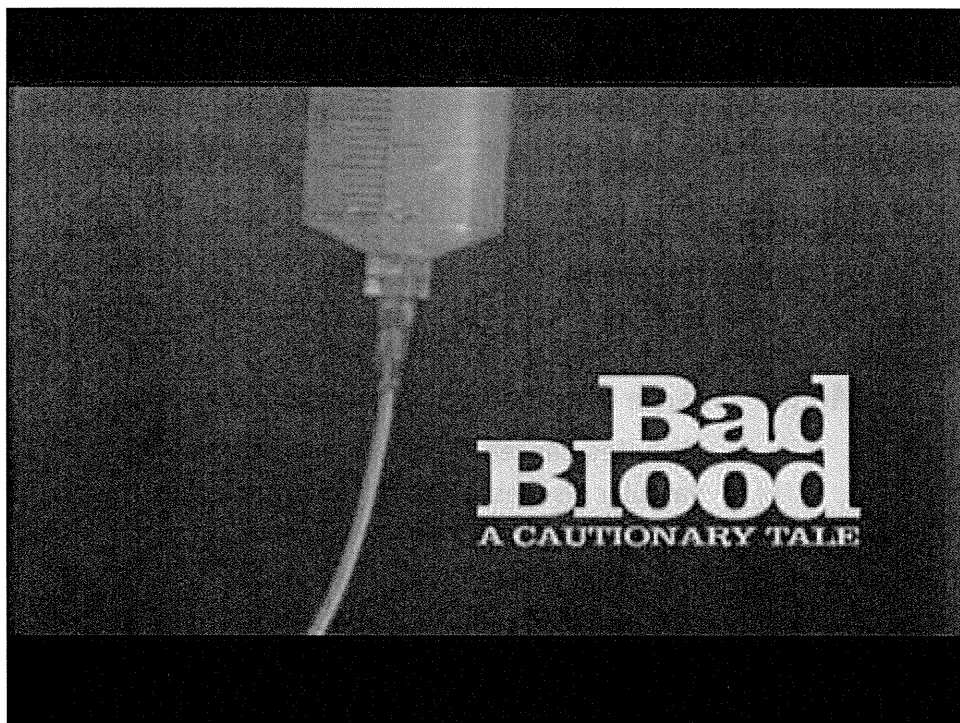
Country	Persons with Hemophilia Who Became Infected, n (%)
United Kingdom	1700 (32)
Japan	1800 (45)
France	2000 (50)
United States	10 000 (50)
Canada	800 (55)†
Denmark	210 (64)‡§

* Except where otherwise noted, data in the table were obtained from Starr (8).

† Data obtained from Kondro (9).

‡ Data obtained from Feldman and Bayer (10).

§ Approximate number.





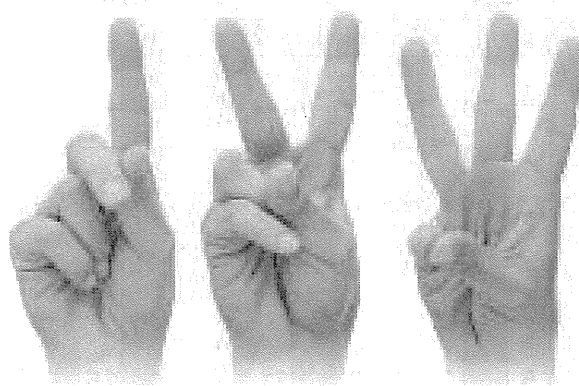
Dutch patient groups

- **Micro** About 400 – 500 disease-specific patient groups
- **Meso** Large disease-specific umbrella groups (diabetes, cancer, rheuma & arthritis, heart & vascular diseases, COPD, neuromuscular diseases and genetic/rare diseases)
- **Macro** The 4 (NPCF, CG-Raad, VG, GGZ, & PGOSupp.)

Worldwide patientmovement

- Similar patterns
- EPF European Patients' Forum
- IAPO International Alliance
 of Patient Organizations
- **Important contacts:** European Commission and
 European Medicine Agency

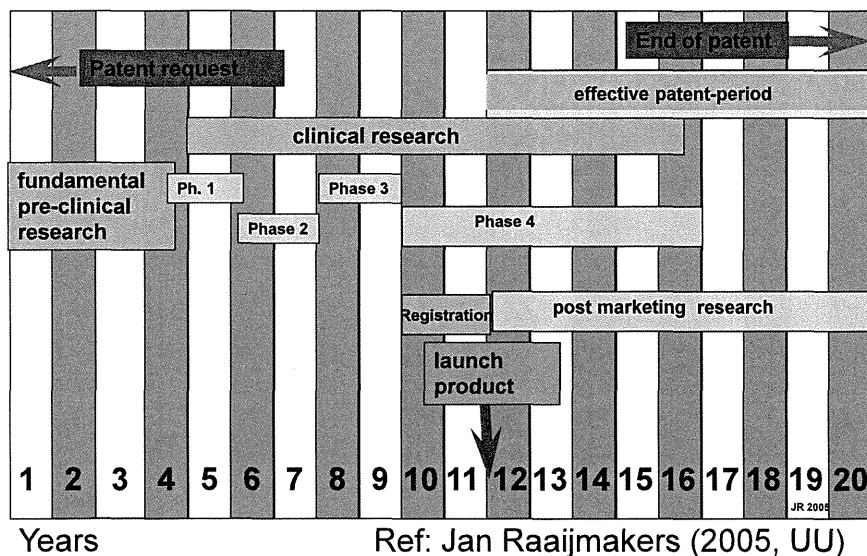
'Third party' role



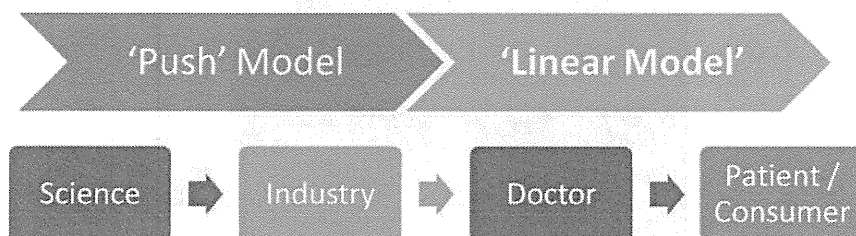
The patient in 40 years

Patiënt 1.0	Information, education Peer support Lobbying for their interests
Patiënt 2.0	Internet, social media, Wiki's, Patientslikeme, SWAN UK
Patiënt 3.0	Cooperation researchers, patients and companies Driving force: 'Unmet medical needs'

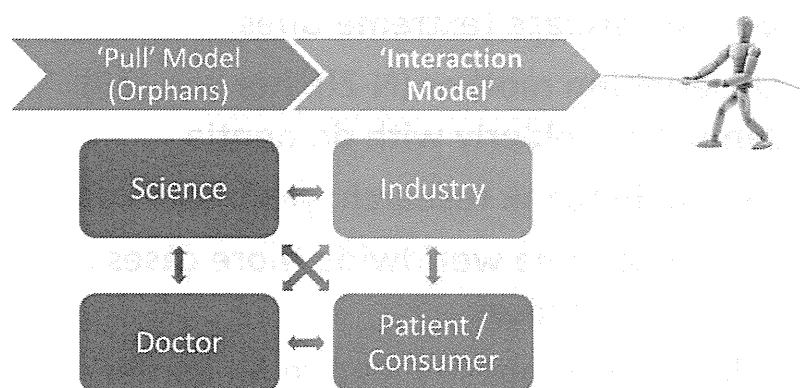
Process of the drug development process

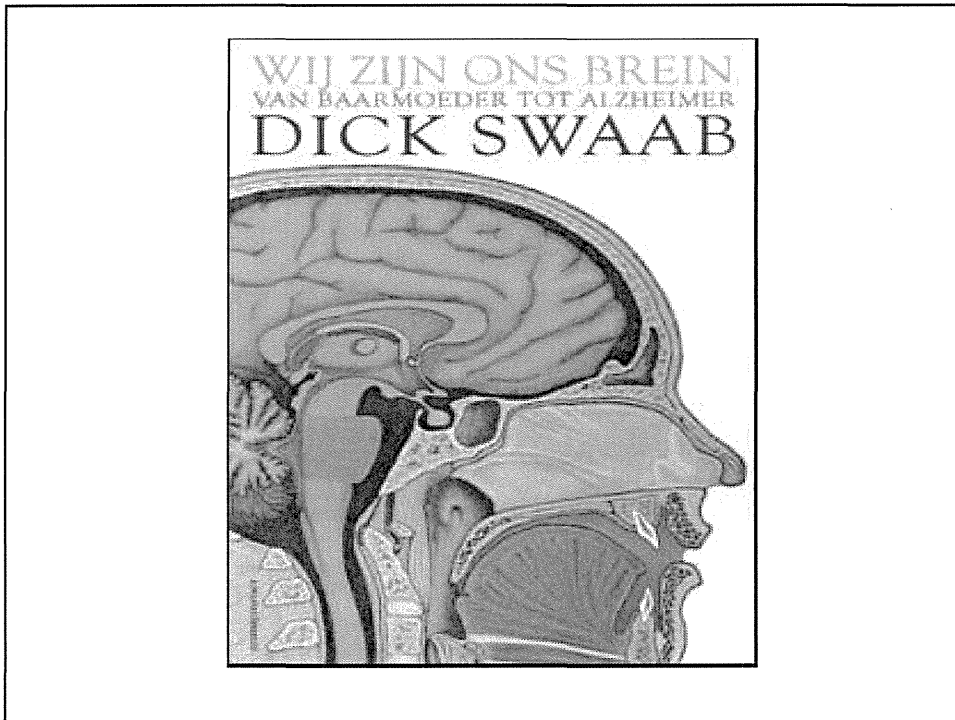


'Duw' or 'Push' model



'Trek' or 'Pull' model





Prader Willisyndroom

- Genetic disease (extreme obese)
- Observation mother (N-ZI), her son (39) symptoms: elderly with dementia
- Swaab, biobank research: yes, more cases
- Since that time worldwide more cases also
General question:
- Will extreme obesity lead to an earlier explosion of dementia/Alzheimer

Patients as driving force (1)

- **Marshall Smith Syndrome**, n = 3 in NL
- Parents of one of these patients started five years ago through the internet a search to other, all patients worldwide
- When they found 17 patients, they could identify one of the genes responsible for MMS (AMC, London)
- Now, ≥ 30 patients who have met each other and have regular contact by skype/webcam

阿克苏诺贝尔慈善登山车队将：
沿着阿姆斯特朗的夺冠之路
挑战环法史上最著名的爬坡段
21道发卡弯 兵家必争之地
实现单日登顶6次

千里走单骑

支持慈善登山车队 捐助全球抗癌基金

六度登顶 极限生存 力克癌症

支持我们的行动
请为阿克苏诺贝尔慈善登山车队募捐！
请为全球抗癌基金募捐！
募捐详情 敬请垂询前台
与我们一起携手帮助“阿克苏诺贝尔慈善登山队”

inspire2live

Alpe d'HuZee

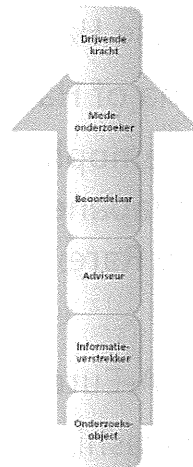
Patients as driving force (2)

- Peter Kapitein (leukemia) & his friends started in 2007 with the organization of a cycle tour to Alpe d'Huez in France
- (6 times up, 6 times down)
- Revenue in 2007: 370.000 Euro
- Revenue in 2012: 32 million Euro
- With 'Live2inspire', they now have an international action plan developed against cancer (support DNB, KNAW) & they have a say at KWF Kankerbestrijding

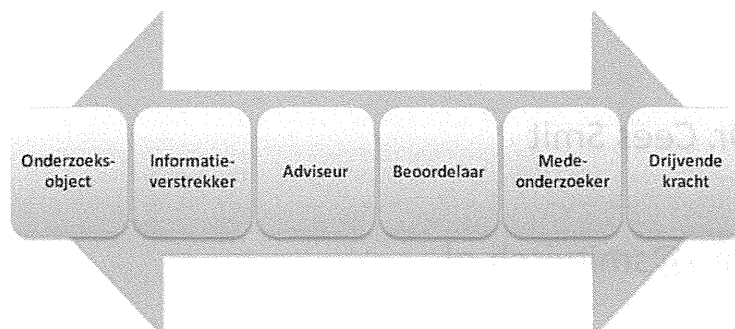
Innovative power patients

- To bring together patients and their body material (DNA, MSS)
- Sharing of data for research and the organization of cooperation (CF/EuroBiobank)
- Fundraising for biobanks and research (gene therapy), also for the long term
(AFM, Généthon, DPP, Alpe du Hu'Zes)
(*Ref.: Van der Valk & Smit, NTVG, mei 2011*)

Participationladder from Arnstein



Participationladder vlg. PatientPartner



Japanese contacts

- Yugo Hanai, Japanese Haemophilia Society
- Ms. Akiko Kakinuma of Habataki
- Ms. Sugiyama of Hemophilia association of Hokkaido Chapter Japanese Hemophilia Society & Japanese Rare Disease Federation

- Mr. Rio Praaning, PA-Europe, Tokyo
- Mrs. Masami Kobayashi, interpreter

For more information

- Dr. Cees Smit

- info@smitvisch.nl

nefarma

vereniging innovatieve geneesmiddelen Nederland

歓迎

Kitasato Research Institute

12th March 2013

The Hague, Nefarma

Nefarma as association

- **Founded in 1975**
- **Focus on innovative (bio)pharmaceutical companies**
- **Condition for membership**
At least 8% of turnover earmarked for R&D
- **Advocacy for big and small companies**

Nefarma as association

Scope and structure:

- **35 members** (as of early 2012)
- **annual budget: approx. €5 million**
- **association with board**
- **supported by an office with a general manager**

Nefarma as association

Representation:

- 90% of turnover from innovative companies
- big and small
- including biotech
- national and international:
VNO-NCW - EFPIA - IFPMA

Nefarma as association

Administrative structure:

- general meeting
- board - 7 members
- office - 19 employees
- working groups (>200 participants)
- consultation groups

Nefarma as association

Advocacy focuses on:

- quick access to market
- fair price-making
- recognising importance of innovation
- sound investment climate
- sound research climate

Nefarma office

Competencies/expertise:

- health care economics
- pharmaceutical affairs
- innovation and biotechnology
- medical-scientific issues
- legal affairs
- communication and public affairs

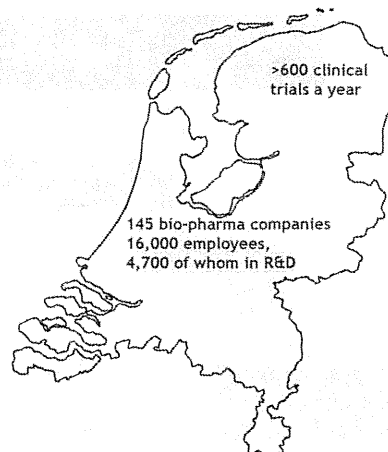
Strategic positioning

Operating in two worlds:

- trade < > health care
- private domain < > public domain
- Means: other norms and values
- Requires: building bridges essential

Strategic positioning

Pharma activity in the Netherlands



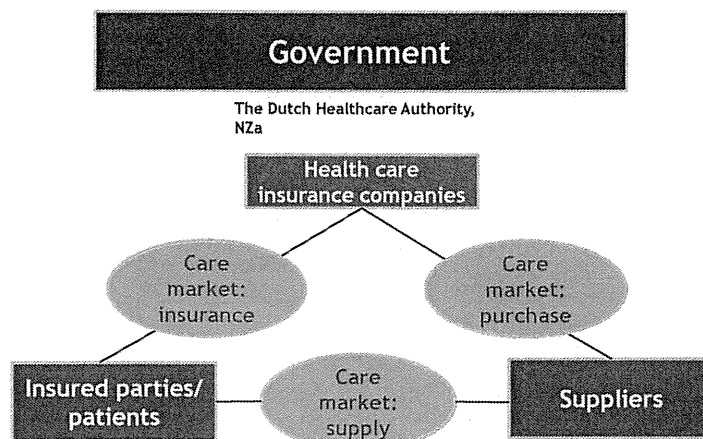
Field of influence

Key stakeholders:

- government and politics
- health care insurance companies
- patients (associations)
- the research world
- prescribers
- the media

Field of influence

Care market in the Netherlands (since 2006):



Field of influence

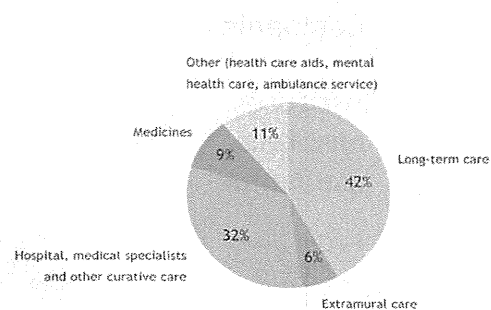
Vision on pharmaceutical care:

- proof of added value
 - substantive
 - price difference with other treatment
- role of treatment guidelines
 - foundation of the *Kwaliteitsinstituut* (Dutch Institute for Health Care Improvement)
- involvement of all market parties
 - incl. prescribers, payers, patients and companies

Medicines sector

Share of health care expenses in the Netherlands

Of a total care budget of €63.5 billion



NB. Incl. distribution in hospitals, medicines share is 10%

Source: National budget, 2011

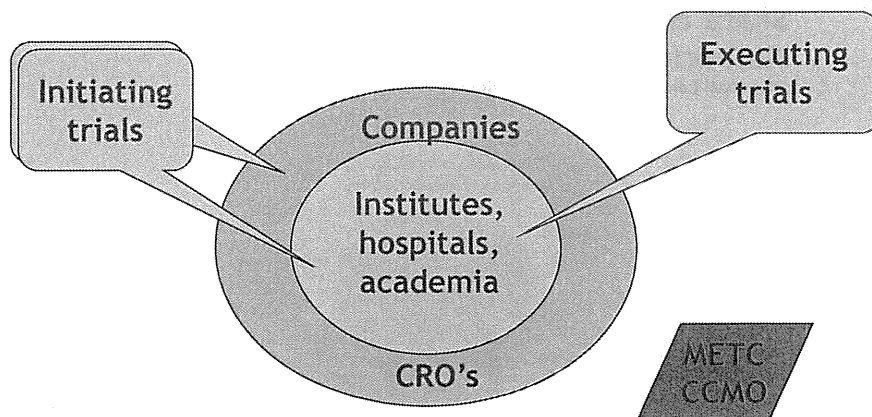
Clinical trials and Nefarma

Internal organisation

- Working group focussed on R&D
medical directors and head clinical operations
- Project group Clinical Operations
clinical operations managers
2013: cooperation with CRO's
- Investment in defining common goals

acron

Conducting clinical trials



Improvement of Clinical Trial environment

- Respect for individual role stakeholder in R&D theater
- Cooperation with other stakeholders is crucial for success:



Initiatives Nefarma

- Expertpanels for improvement R&D area
- External Review Directive 2012 by CCMO
faster submission of the protocol
- Congress Royal Netherlands Academy of Arts and Sciences: focus on improvements



Masterplan Dutch Clinical Trial Foundation
project manager:
– facilitated by Ministry of Health
– DCTF responsible