# The added value of Inno-HTA for consumers/patients

Dr. Cees Smit, VSOP/EGAN

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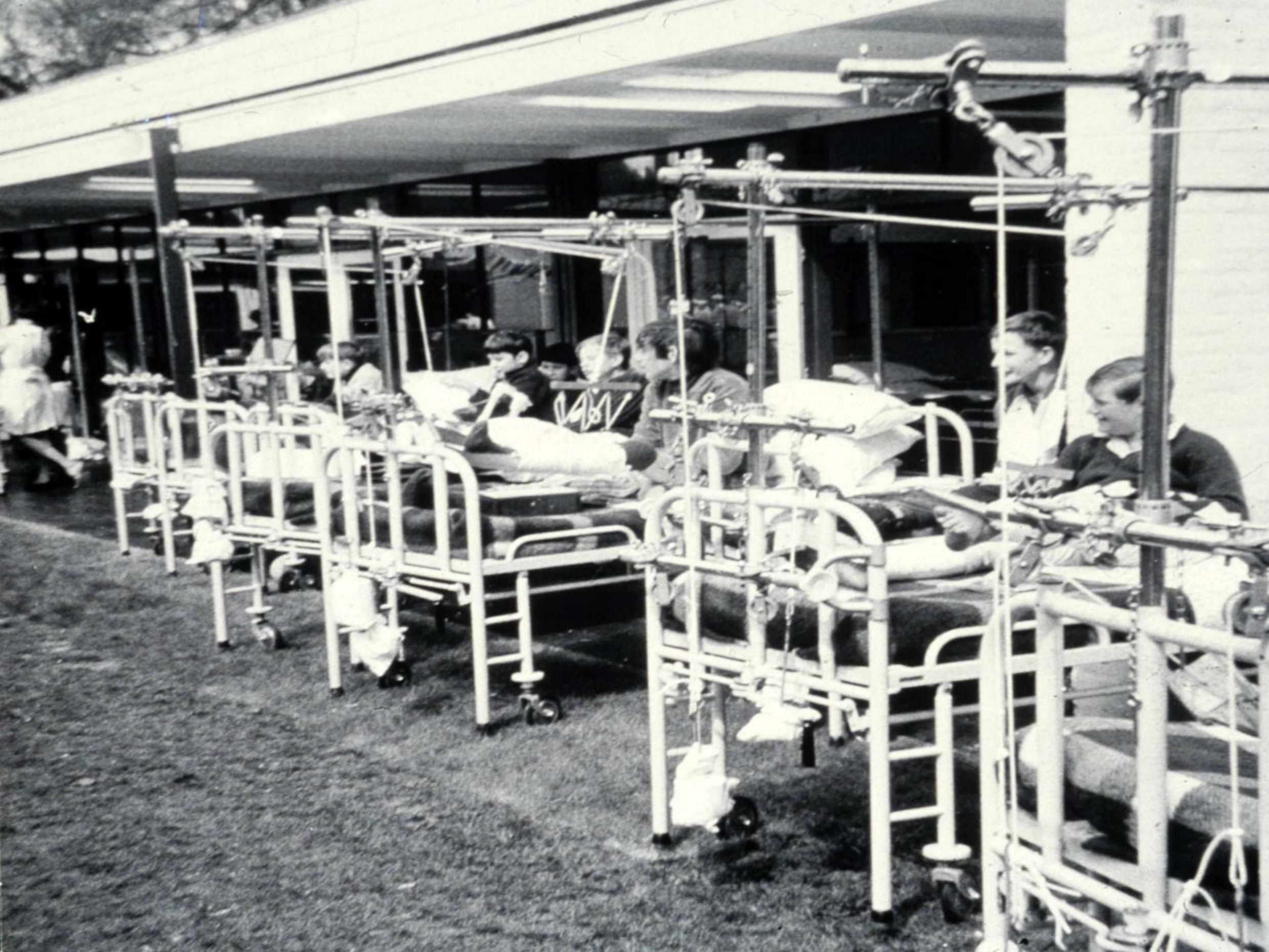
# Topics

- My personal background
- Dutch Haemophilia Study
- The Inno-HTA project
- The Patient/Consumer perspective
- Value to whom ?
- Healthcare Value Model
- Problems with QALY's
- Patients as active researchers

# My personal background

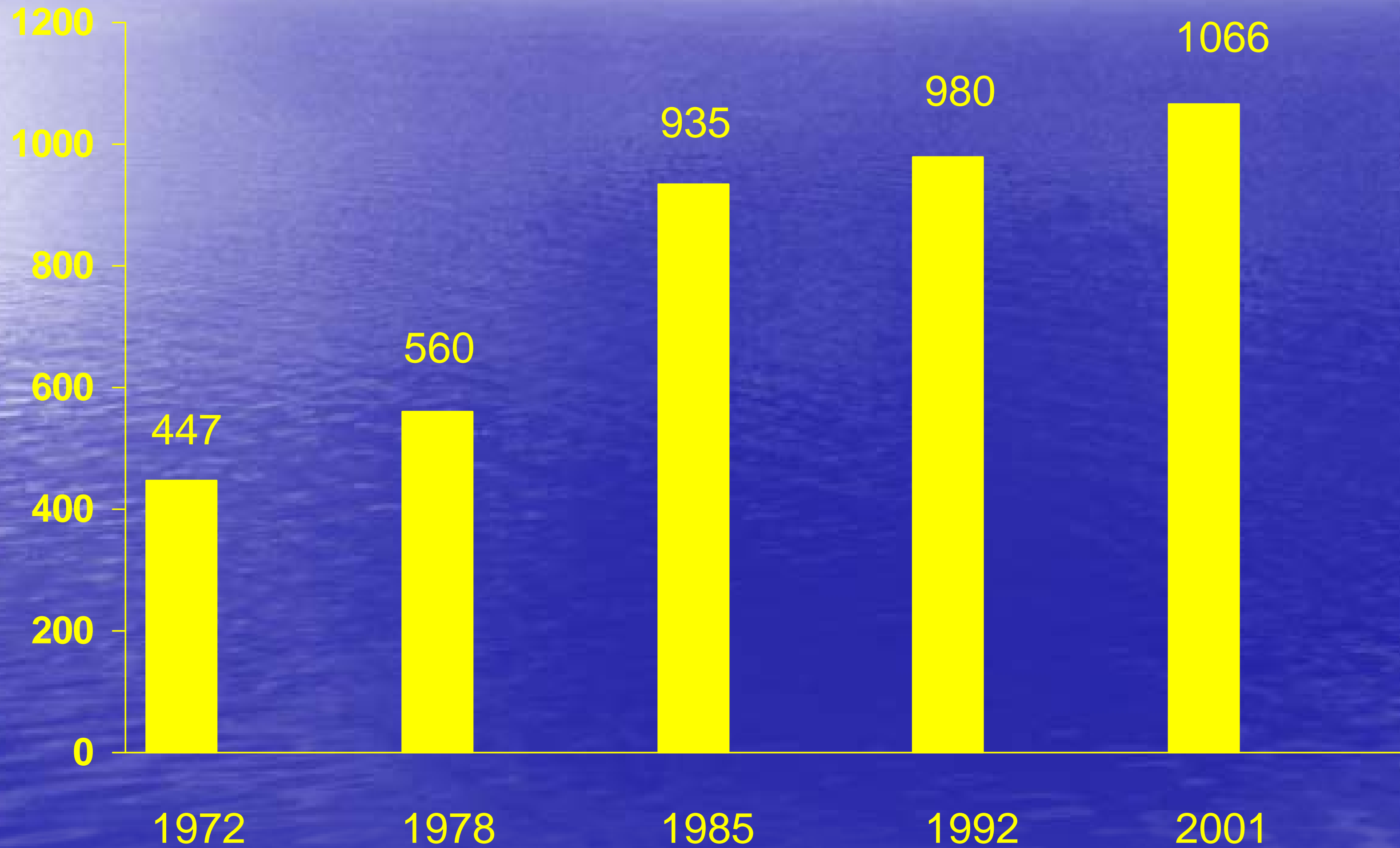
- Severe haemophilia A
- Studied business economics
- Active in patient groups since 1971
- Co-ordinator Dutch Haemophilia Society
- VSOP, Dutch Genetic Interest Group
- EGAN, Eur Genetic Alliances' Network
- April, 2008, member CvZ Dutch Appraisal Committee (Health Care Insurance Board)



# The Dutch Haemophilia Study

- 1965/70, no treatment for haemophilia
- ~ 1970 start of treatment
- 1971, initiative for a long-term study to study the effect of treatment (med/social)
- Since 1971, five mail questionnaires
- High response rates
- One of the few large follow-up studies on effect of treatment for a chronic disease

# Participants



# Haemophilia

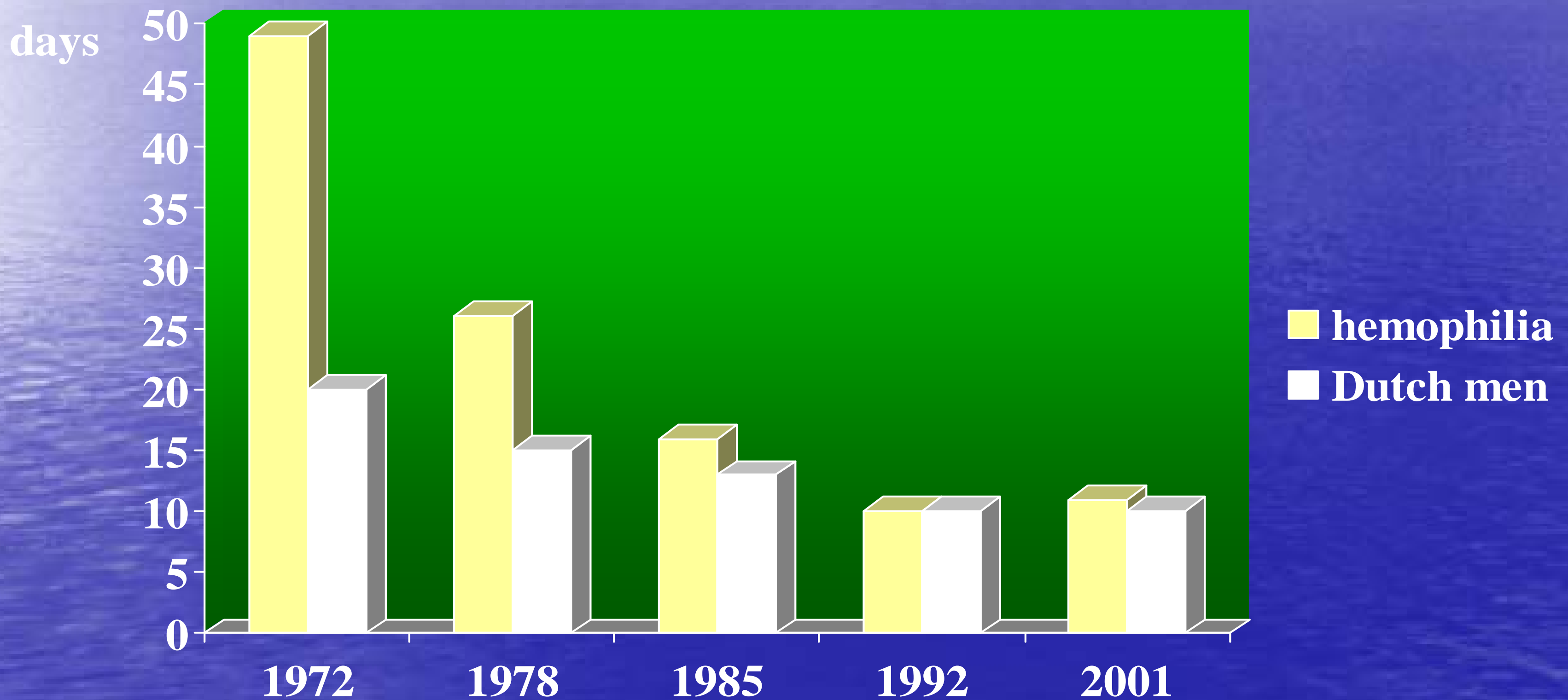
- Average age (anno 2007: normal life-expectancy)

| | |
|---|---|
| 1972 | 22 |
| 2001 | 32 |

- Average age of men with haemophilia at the start of inability to work

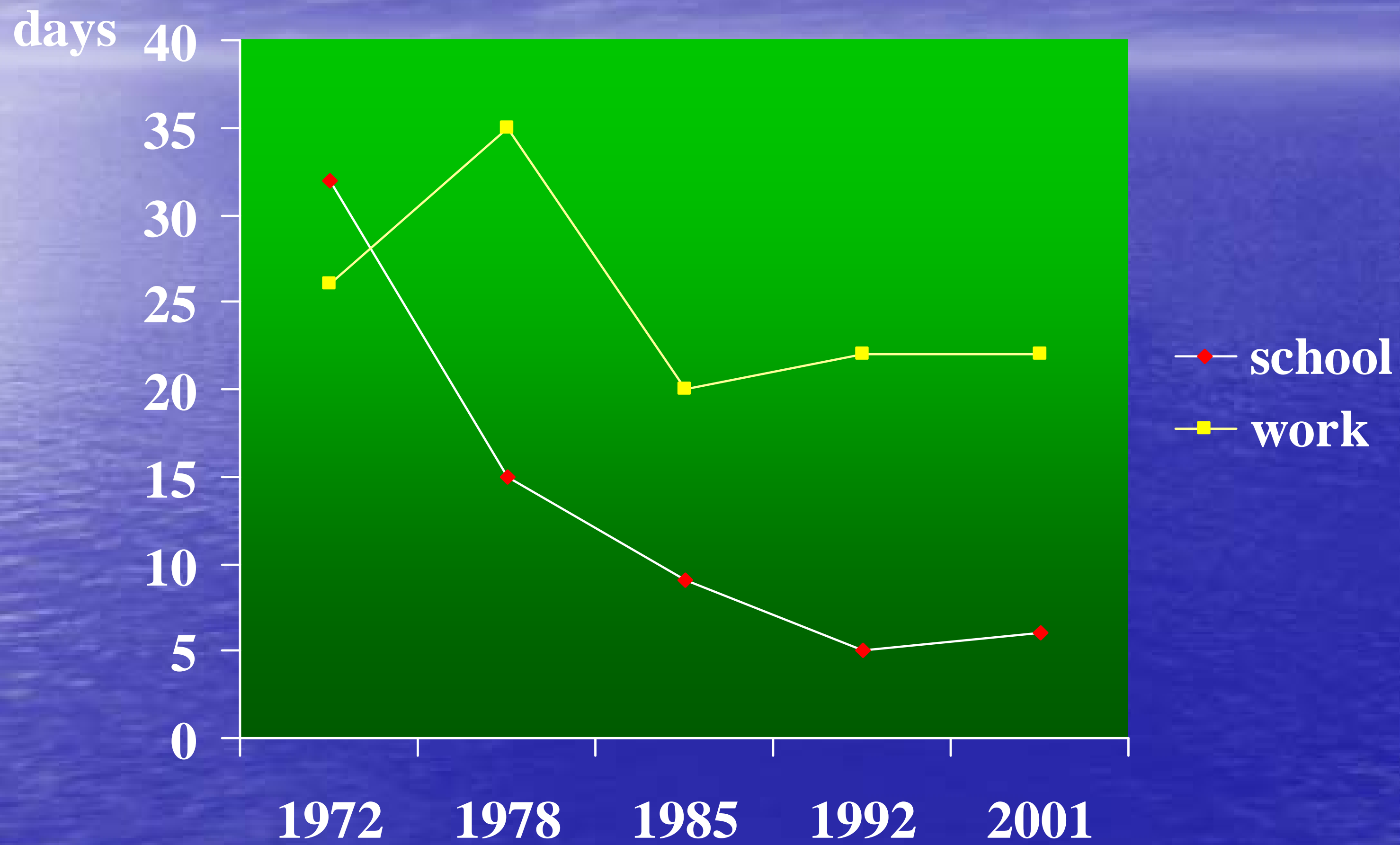
| | | |
|---|---|---|
| 1972 | 32 | |
| 2001 | 49 | Leiden, HiN-5, 2004 |

# Duration of stay in hospital
## -severe and moderate hemophilia-

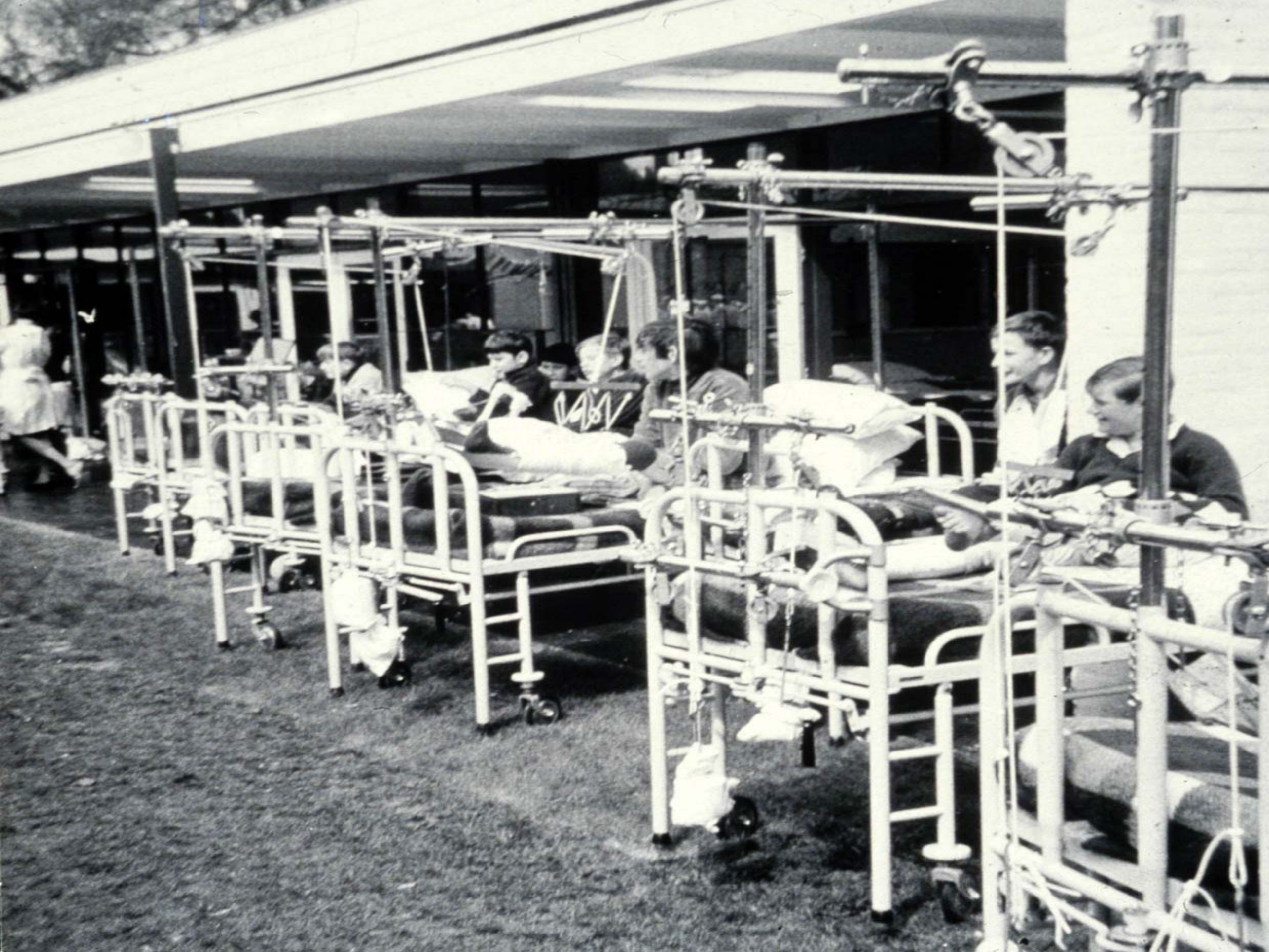



HiiN-5

# Absenteeism from school and work




HiiN-5



# Inno-HTA Project (1)

- Very positive development to look to  innovation in HTA (62 indicators)
- Important indicators:

  1-10; 22; 32; 35-38; 41-43; 45; 49-50; 60
- Evaluating health care innovations at an early stage is absolutely necessary and is often not happening or refused (e.g. health care insurance reform in NL, RGO or labour reintegration, NL)

# Inno-HTA Project (2)

- Inno-HTA important additional tool at the beginning of the thinking process
- HTA by patients, now often perceived as the QALY-concept and it comes at the end of the process
- At that stage it is very difficult to give input from a patient perspective
- Will it lead to other decisions (go–no go)?

# Inno-HTA Project (3)

- Who are the stakeholders ?
- Who’s the patient ?
- Who’s the consumer ?
- What’s value ? To whom?

- Interactive HTA (Reuzel, NL)
- prioritizing studies (Broerse, Abma, NL)

# Who's the patient, the consumer?

- In health care:
- The consumer: low premium
  high own risk/
  self payments
- The patient: Who's the best doctor ?
  What's the best hospital ?
  What's the best medication ?

# Who's the patient, the consumer?

- Different attitudes on new technologies, e.g. in health care with regard to:
- Genetic modification
- Nano(bio)technology
- Animal experimentation

- Good, balanced stakeholder representation needed, how to organize ?

# Who's the patient ?

- Development of cochleair implants
- Technology not accepted by the deaf community, denial of their own culture, sign language

  (Blume, Reuzel, NL)
- Similar developments with regard to prenatal diagnosis, embryo selection (parents who want a blind child)

# Value to whom ?



- Value to individual patient: treatment with most health benefits
- Value to healthcare system: treatment with best health benefits relative to costs
- Value to payer: treatment with lowest cost
- Value to society: treatments with health and social benefits relative to other uses for public funds



(Durhane Wong-Rieger, CORD, EHFG, 2008)

# Healthcare Value Model



| Value to Funder | Social Value |
| --- | --- |
| **Desired Outcome**<br>Cost-effective<br>**Driving force**<br>HTA to support cost containment<br>**Principle**<br>Least cost | **Desired Outcome**<br>Most access to all<br>**Driving Force**<br>Informed public, expert support<br>**Principle**<br>People's Choice |
| **Individual Patient** | **Patient and Health System** |
| **Outcome**<br>Treatment to those in need<br>**Driving Force**<br>Need for treatment, rescue<br>**Principle**<br>Rule of rescue, individual good | **Desired Outcome**<br>Most treatment<br>**Driving Force:**<br>HTA to support most patient access<br>**Principle**<br>Level Playing Field |

Value to Payers (vertical axis)

Value to Recipients (horizontal axis)

# Healthcare Value Model

- HTA to support cost containment

  versus

- HTA to support most patient access

- Most European patient groups rather critical of HTA/QALY-approach, because QALY's are perceived as life-threatening and patient values as undervalued

# Cost of haemophilia treatment

- Higher than in most QALY-discussions (range of 20.000 – 80.000 Euro/QALY/year
- Severe haemophilia: 130.000 Euro
- Average for all people with haemophilia in the Netherlands: 37.500 Euro

# Is reflection possible ?

- Look to the development of a haemophilia treatment retrospectively with:
- Inno-HTA Approach
- The QALY-concept
- Hurdles in the treatment process (Viruses, need for safer technology, recombinant technology)
- Results 'Long term outcome' study
- Need for cheaper technology (industry, patients)

Zinnige en
duurzame zorg

Zicht op zinnige
en duurzame zorg

Rechtvaardige en
duurzame zorg

# Content RvZ-reports

- the Council for Public Health and Health Care (RvZ) addressed the issue as to which criteria should be applied in order to identify priorities for the funding of care from collective resources
- In the media: A maximum QALY of 80.000 Euro
- The report left two key questions unanswered: what constitutes 'fairness' and 'what roles and responsibilities do the various stakeholders have in the prioritisation process?'

# Evidence Based Medicine

- Originally, combination of three elements:
- Goal of the treatment (needs and wishes of patients)
- Proof of effectiveness of treatment
- Clinical expertise of medical professionals

- Now, EBM/QALY is moving to an economic goal, to limit the costs of health care

# Problems with QALY's (1)

- Some costs & benefits are overlooked
- Long-term informal care from parents
- Loss of career-perspective from one of the parents
- Long-term loss of labour-productivity
- Long-term loss of tax-payments
- People with diseases have less to spend

# Problems with QALY's (2)

- If few people use specific care or medication, this will lead to a higher price
- If more people use specific care or medication, this will lead to a lower price
- QALY-instrument less effective to take into account the frequency of a disease

# Ethics and QALY's

- What to offer a patient, when society finds a treatment too expensive
- Refusal of treatment is a death-penalty
- Requires hospitalization: 100.000 Euro

# What is often overlooked

- People in the EU value good health care (Sweden/MPS, Nice Citizen Council 2004)
- EU wants to stimulate development of orphan drugs, MS have cost problems (driving a car: accelerate and brake)
- Health as an investment in society: Employment, knowledge economy, etc.

# Role of patient organisations

- **Three traditional roles:**

  **to provide information**
  **to offer & organise peer support**
  **to play an advocacy role**

- **A fourth, new role:**
  **participation & consultation in research through 'Experience Based Knowledge'**

# Patients as entrepreneurs: PXE

- Sharon & Patrick Terry, two kids with PXE
- In 1995 they started in the USA to work on fundraising & organizing the research to find the cause of PXE and the road to treatment
- A non-profit company, 52 offices/world
- Within 10 years material from 2.000 PXE patients was collected and analyzed
- They found the gene 'ABCC6' for PXE
- The patent rights of the ABCC6-gene were assigned to PXE International

# Patients & HTA-education

- There is in Europe a need to train patient groups in HTA-methodology and challenge these concepts against their own ‘real life’ impact and long-term outcome studies
- ‘Understanding HTA’ of Health Equality Group (June 2008), by Karen Facey
- HTA Summer course, Eurordis, 2010
- Inno-HTA also here an important tool

# Real life impact

'When I started on MPSII Enzyme Replacement Therapy, in the six minute walk test I could walk 16 metres. At my six minute walk test last week I could walk 257 metres and further if I hadn't ran out of time. ERT has changed my life!'

- Writing a letter, comb my hair, etc.

# Conclusions

- Very positive development to look to the innovation component in HTA
- Inno-HTA important additional tool
- Better starting-point for discussions with European patient groups than the actual discussion on QALY's
- Increasing tendency within patient groups to engage in research & fundraising

# For correspondence


- smit.visch@wolmail.nl