International lessons from a Dutch reimbursement procedure on Pompe and Fabry disease

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Ede, ICORD, October 9, 2014

Declaration of interests

- Member of appraisal committee of the National Health Care Institute Package (ZiN) since 2008
- An expensive patient myself (hemophilia)
- Written several books for pharma companies, on hemophilia/hiv at a refund of cost basis
- Attended a meeting from Shire, June 2013

Dutch television, June 2012

Wat mag een jaartje langer leven kosten?

In ieder geval geen 400.000 euro, vindt het College van Zorgverzekeringen. Het College adviseert nu om 'te dure' medicijnen voor zeldzame ziektes niet meer te vergoeden. ► pagina 4&5

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Half 8 Live: Nieuwsuur *meets* Knoop in je zakdoek

► pagina 17

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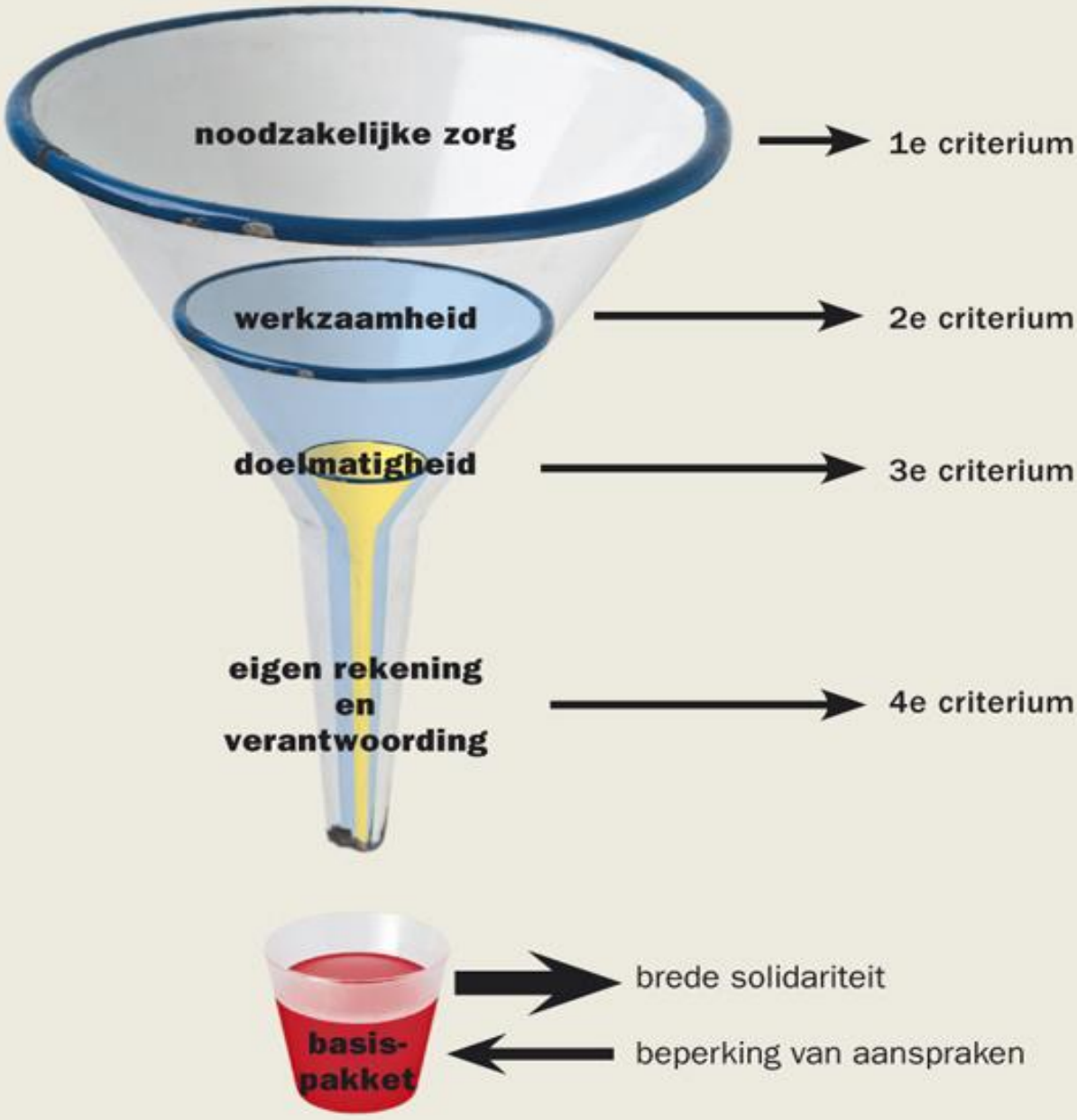
History of package criteria

- Trechter (Funnel) of Dunning, 1991
- Reports in 2006/7 (RvZ) sets a QALY criterium per year of 80.000 Euro (NICE, 20 – 30.000 Euro)
- ZiN (Dutch Health Insurance Board) advises the Minister of Health (VWS)
- They used for the first time cost-effectiveness as final criterion for patients with Pompe and Fabry (summer 2012)

De trechter van Dunning



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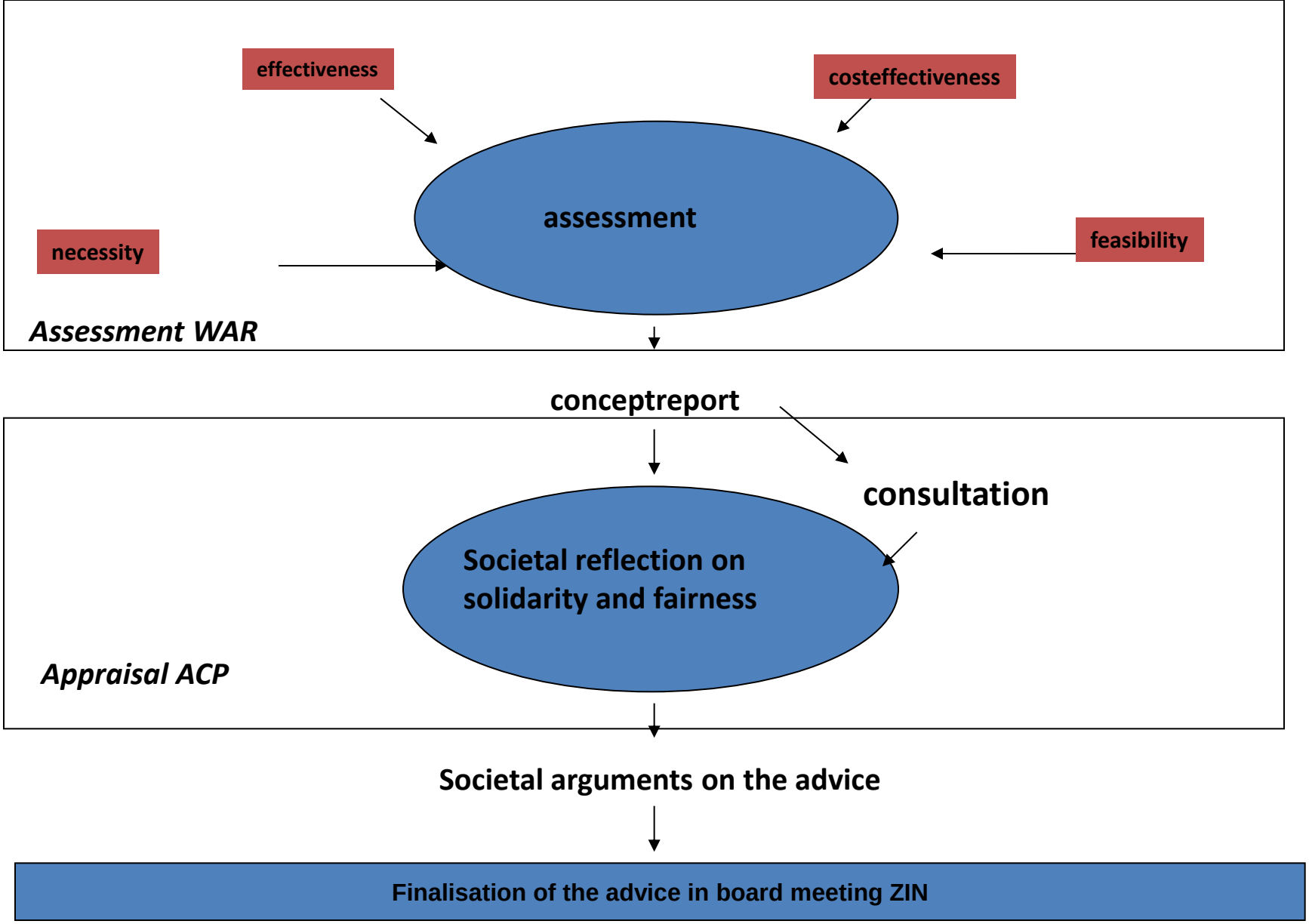
Pompe and Fabry's disease (1)

- **Several rounds of judgments and financing methods in the ten years before 2012**

- **T = 4 judgment (2006/7 – 2011)**

- **Technical Assessment WAR/CFH, (written comments)**
- **Desk-advice ZiN staff people**
- **Societal judgment ACP**


effectiveness
costeffectiveness
assessment
necessity
feasibility
Assessment WAR
conceptreport
consultation
Societal reflection on solidarity and fairness
Appraisal ACP
Societal arguments on the advice
Finalisation of the advice in board meeting ZIN

Pompe and Fabry's disease (2)

- **WAR/CFH: Therapeutical added value (2/3)**

- **Desk-advice ZiN June 2012:**
- **Limited average effect**
- **Cost-effectiveness very bad**
- **Don't include in basis insurance package (2/3)**

- **ACP: Societal judgment on September 21, 2012**

Public Hearing, September 21, 2012


Eekholt 4
HET WERKT bij mij
FSIGN
Minister
Het werkt.


HET WERKT
bij mij

FSIGN

Fabry Support & Informatie Groep Nederland

Minister,

Het werkt....

Ik ben te duur

en krijg de

doodstraf

Outcome of the public hearing

- Impressive input from the physicians treating Pompe (Ans van der Ploeg) and Fabry (Carla Hollak) and the patients
- Also from Yann Le Cam, representing Eurordis & the international patient community
- Decision much more positive: Minister continue treatment (through a separate fund and not from the basic insurance package)
- The Minister promised to pay another 2/3 years

Lessons learned (1)

- ***Structure (1)***

- **Patients made a *s*trong and urgent plea to deal with this type of reimbursement procedure on a European level. This was also addressed by the treating physicians.**

- **Physicians also stressed the need for more data and unified patient registers on an international scale.**

Lessons learned (2)

- ***Structure (2)***
- **There is no coherent structure within ZiN to allow such a debate in an early stage**

- **There is no possibility to merge the 'hard' criteria of HTA assessments with the more 'soft' considerations of moral factors such as ethics, equity, society views, etc**

Lessons learned (3)

- *Discontinuation of treatment*

- **This will cause costs as well. In the long run Pompe patients need continous breathing assistance and an earlier death will follow**
- **Larger costs of informal care**
- **What can society offer a patient when he refuses such a state of detoriation?**

Lessons learned (4)

- ***Access to orphan drugs***

- **Great success of US Orphan Drug Act (1983) and EU Orphan Drug Act (1999)**
- **But there is no clear policy on how to grant access within reimbursement procedures**
- **Is there a conflict with Universal Health Care and Right to Health concepts?**

Lessons learned (5)

- ***Pricing***

- **What is a realistic price for an orphan drug that allows industry or other producers (patient groups) to earn back its investments in new products/treatments?**

Recommendations (1)

- *Consultation*

- **Patient groups should understand the importance of timely consultation, as well for registration as for reimbursement procedures**
- **Other stakeholders should work also according these lines (EMA and FDA do), but HTA authorities/ethics committes lag behind**

Recommendations (2)

- ***Outcome measures***

- **Patient groups should develop their own relevant outcome measures, like in arthritis and Duchenne research**
- **Make short movies with patient stories for agencies**

Recommendations (3)

- *Funding*

- **The patient community often lacks sufficient resources to be fully equipped to play a serious role in the consultation processes**
- **There is no clear policy from the EU or EU MS how patient groups should fulfill their 'third party' role in health care (esp. funding)**

Recommendations (4)

- ***Education***

- **Stakeholders should learn from each other about good practices in active patient participation on research & policy activities**
- **Patient groups could learn from projects like Value+, PatientPartner and EUPATI**

Recommendations (5)

- *Price*

- **The estimation of a realistic price for orphan drugs and pers. medications & diagnostics**
- **The massive burden of legislation can be reduced without comprimising safety**
- **Risk-benefit scenarios should be revised**

For more information

Ref.: 'Personal Reflections of a Patient Representative in an Appraisal Committee'

Online: www.link.springer.com/journal/40271

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