



Zorginstituut Nederland



## Registries: oase of fata morgana?

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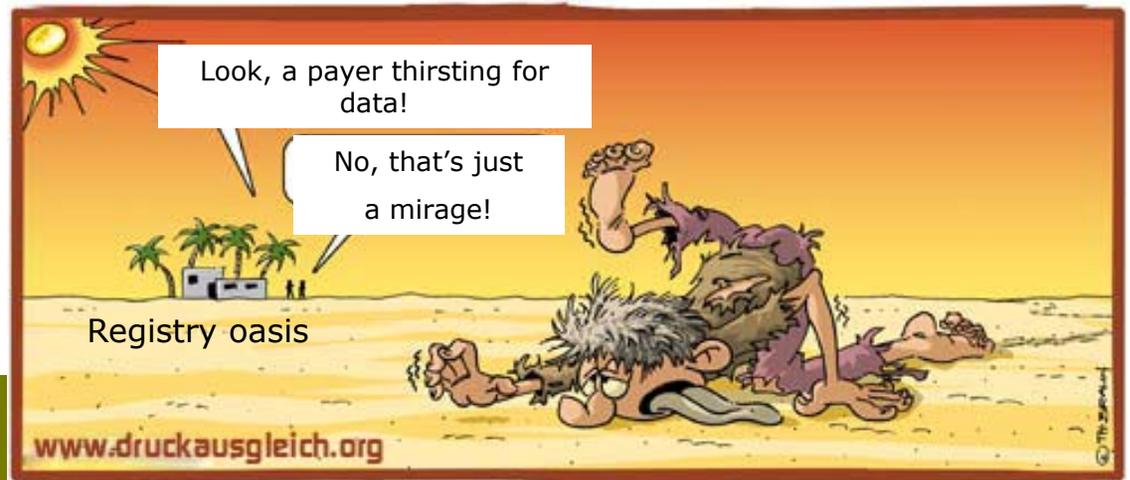
## Basic problem of reimbursement

- Problem: Investment known at reimbursement decision, ROI may materialize many years later
- Value = balance between investment in patients and its ROI
- Value  $\neq$  cost effectiveness
- Solution: "Big Data": but should lead to decisions that are respected by all, or else will backfire



Perceived solution: registry-based outcomes research but with generally known shortcomings

- Incomplete data
- Changed treatment paradigms
- Long-term endeavor so protracted delay in decision making





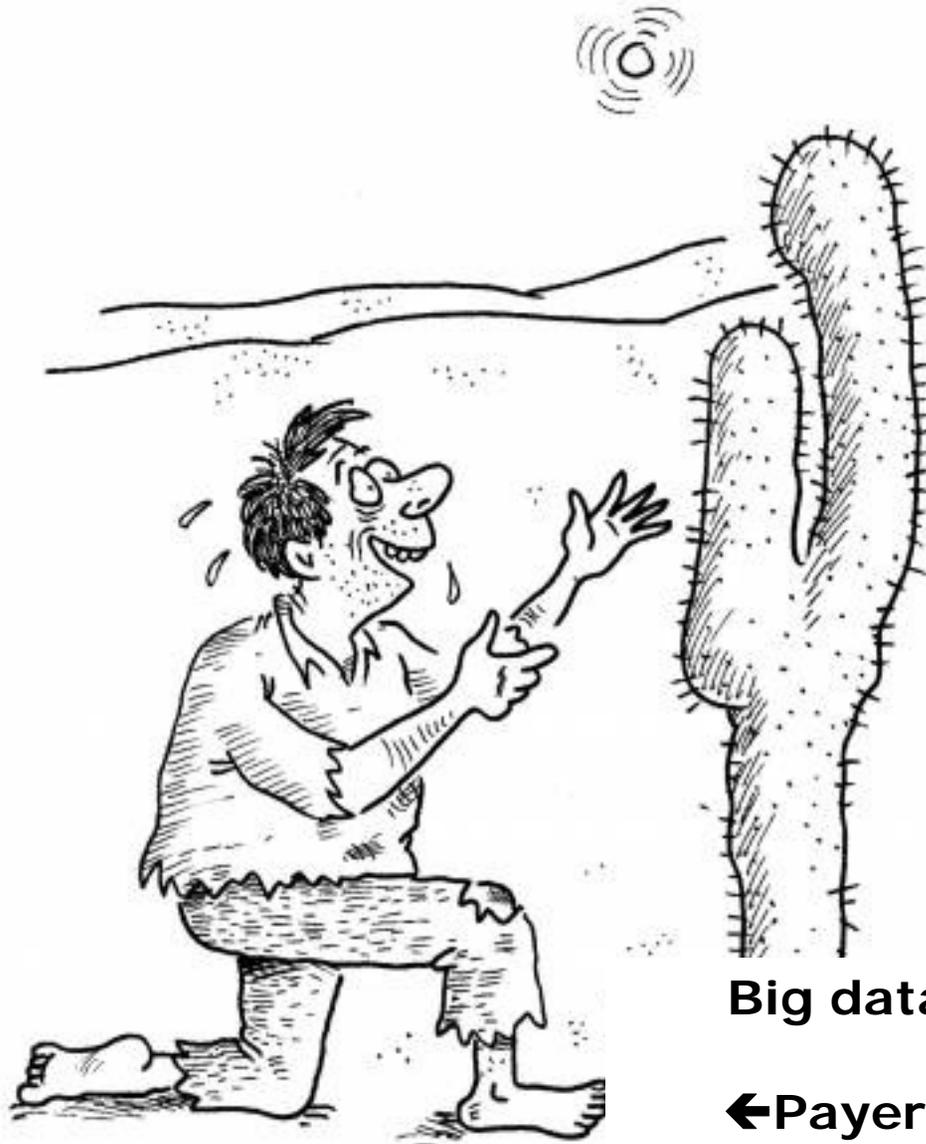
The more you need big data & registries output to inform decisions, the less you are likely to have them

- Many new orphan drugs tend to be registered by EMA more on the basis of possible future usefulness than solid evidence
- Hope: RWE fills the gap in due time
- Single interventions in a chain of oncology treatments: how will we ever be able to discriminate between their contribution to overall - or progression free- survival, and the contribution of other interventions?
- An increasing number of combination treatments will become a trending topic which further decreases the likelihood that registries will inform payer's decisions



## Importance of RWE, registries and Big Data

- Big Data useful in situations that do not change rapidly, less useful in negligible or extreme treatment cost
- Registries should be shaping practitioners' awareness of what they are doing, more than generating evidence to justify policy decisions to be taken by payers
- Big Risk of Big Data and registries: creates *again* the giddy prospect that if you do not possess the relevant data at the agreed timepoint, the Real Answer may still materialize magically some years later.



**Big data**

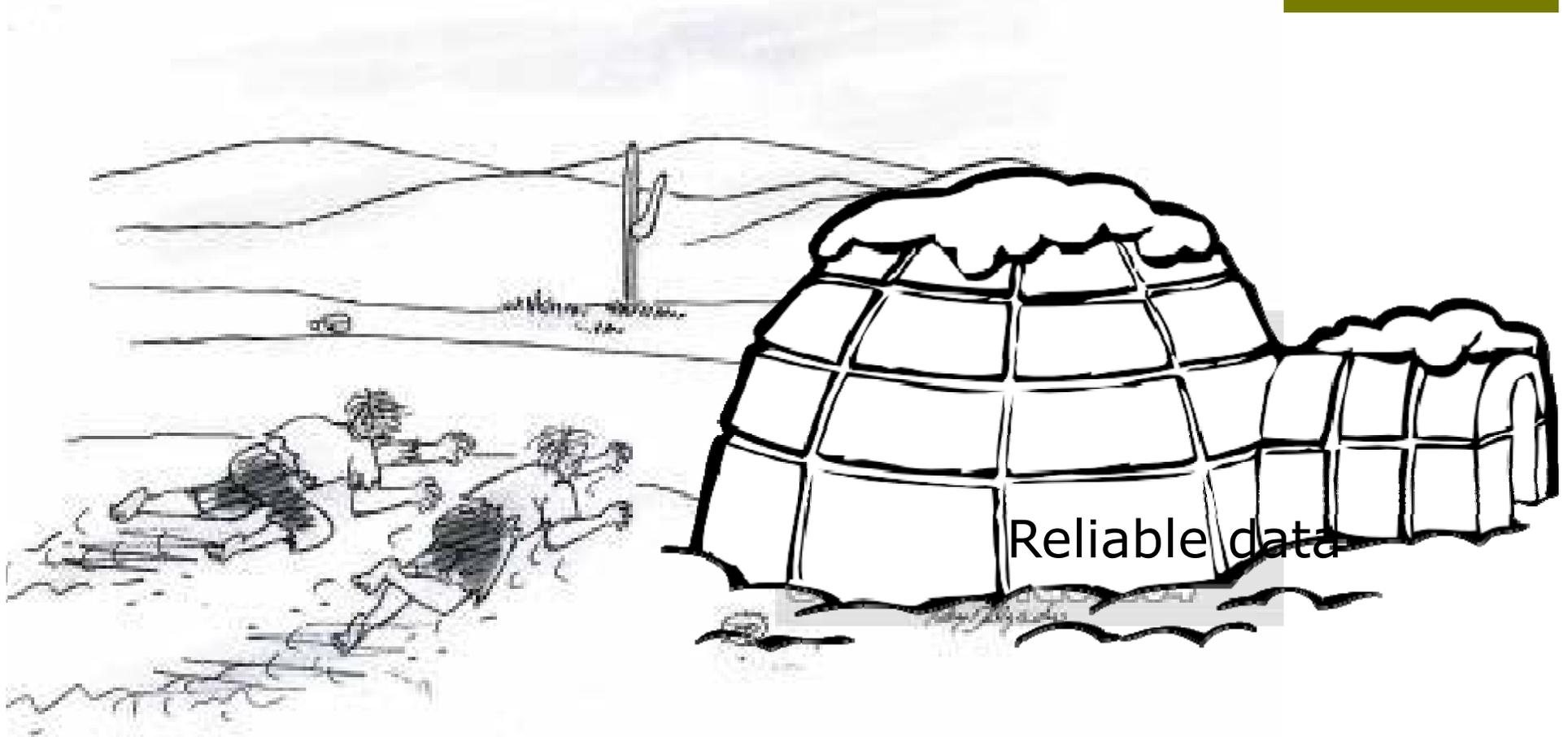
**←Payers**

"Darling, may I have your hand in mirage?"



## Limitations of Big Data we must live with

- Technology is awesome but: watch out for technology drive
  - Eg easily measured but useless surrogate endpoint
- Garbage in = garbage out, even doctors do not always enter data accurately
- Software systems for measuring e.g. tumor diameter rarely agree
- Patient powered research eg: [quantdoctor.com](http://quantdoctor.com), [patientslikeme](http://patientslikeme.com), [quantified self](http://quantifiedself.com), are all great but risk of “selection” bias
- Gamification invites cheating

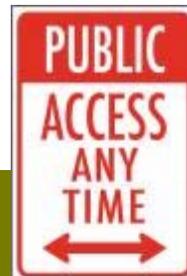


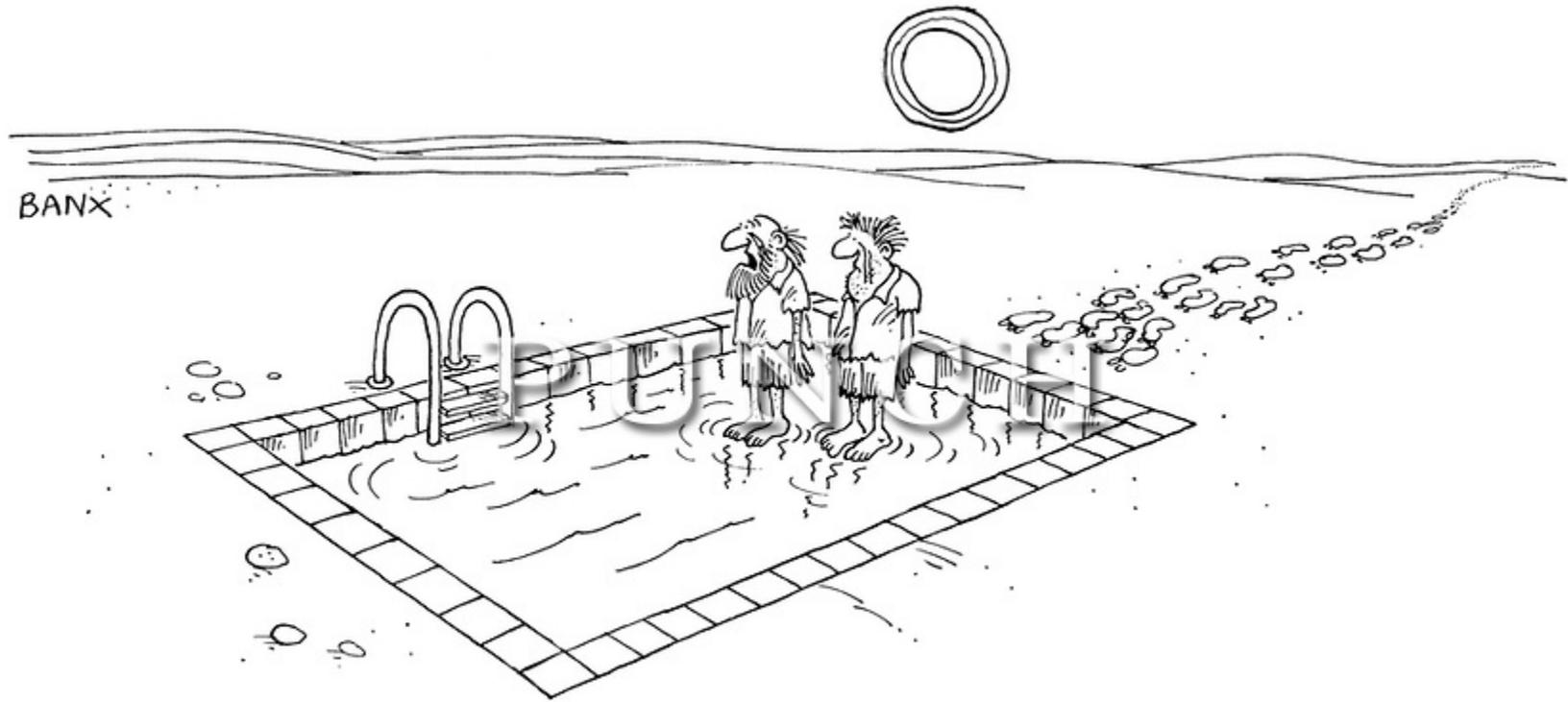
***" It must be a mirage. "***



## The RWE/registries avalanche

- RWE mostly collected for what industry & prescribers think relevant
- Informs generally more about safety than effectiveness
- Contains generally no good QoL (or ASCO-like params.)
- Or other parameters that help payers decide (EDSS)
- Disease based, EU wide,



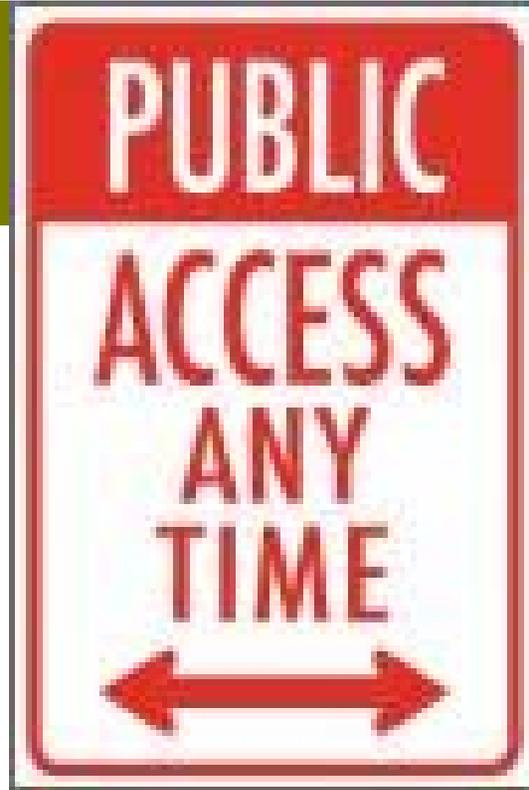


*"As mirages go, it's a tough one."*



## The vested interests

- EMA demands registry from industry for a product. Getting access to data is very hard
- Number of registries in the Netherlands managed by third parties, who generally do a good job but have a certain need to prove themselves indispensable
- Doctor-driven registries frequently in cooperation with university which means delayed public access or no access at all.



It is data on drugs paid for with public money, so unhampered public access should be a right for all HTA institutions

So the ownership question is less interesting than the access question



## Use of registry for payers (1/2)

Will the registry:

- include an outcome parameter that is easily measured and correlates with detectable patient improvement?
  - dare we use modern technology to collect unfamiliar types of evidence?
- help discuss acceptable cost/outcome upfront?
- show to what extent the use of a given intervention is warranted /necessary?
- Shrink bandwidth around CE estimate?



## Use of registry for payers (2/2)

- enable insurers to orchestrate the use of the product?
- promote reporting on (enforcement of) start/stop & dosing changes
- foster dialogue between patients, doctors and payers about rational use?
- yield actionable data within a year or: act on accumulating data in real time?



## Big Data Lookout

- Scientific certainty, obtainable some time in the indeterminate future, gives a solid reason for possibly abrupt change of course
- Less solid data from real life feedback using all possible rapid reporting means, including patient diaries and data from wearables,
- Should be considered as a means to more flexibly correct payer course in mid-stream.
- It is a trade-off: certainty when perhaps too late, replace by a likely estimate earlier on.



Big data deliverables?  
Third mirage on the left!



## Wat is het IMI-GetReal Project?



scope:

- Europees

Hoofddoel:

- Mogelijkheden voor het gebruik van real-world data uit de klinische praktijk in het ontwikkelingsproces en beoordelingsproces van geneesmiddelen
- WP1: opbouwen van een policy framework
- WP4: best practices voor statistieke methodes, ITC, data synthesis



## Role of ZIN

Less of an arbiter, more of a broker

That facilitates cooperation between stakeholders

In the interest of both patients and other insured citizens



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