NICE National Institute for Health and Care Excellence

The patients understanding of benefit risk

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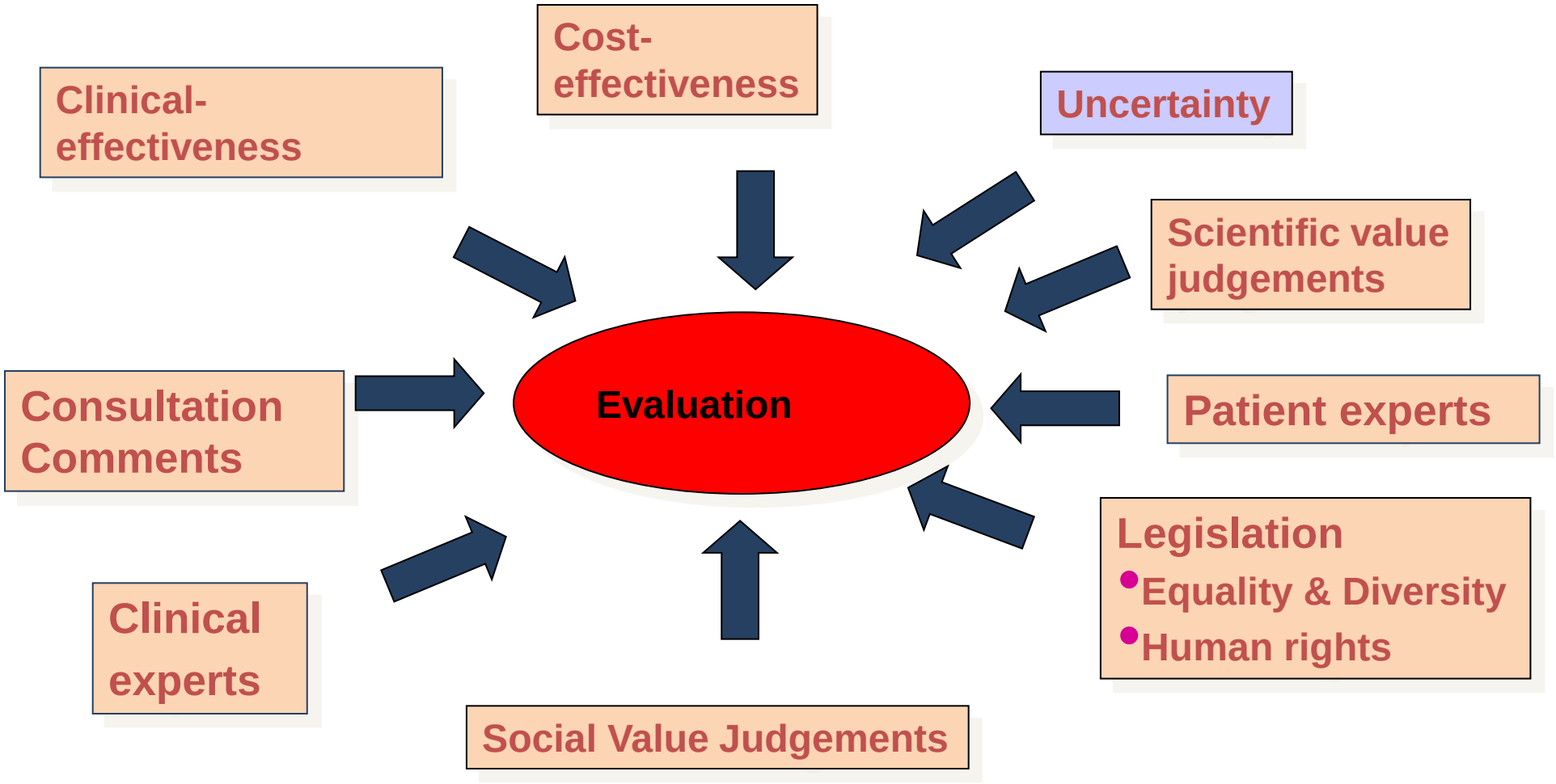
What gets taken into account?



What type of uncertainties?

- clinical effectiveness
- cost effectiveness
- implementation
- outcomes
- accuracy of a test
- clinical prediction rules

- diagnosis
- prognosis
- rates of harms
- patients' experience
- measurements of outcome
- service delivery
- organisation
- Methods

Types of uncertainty

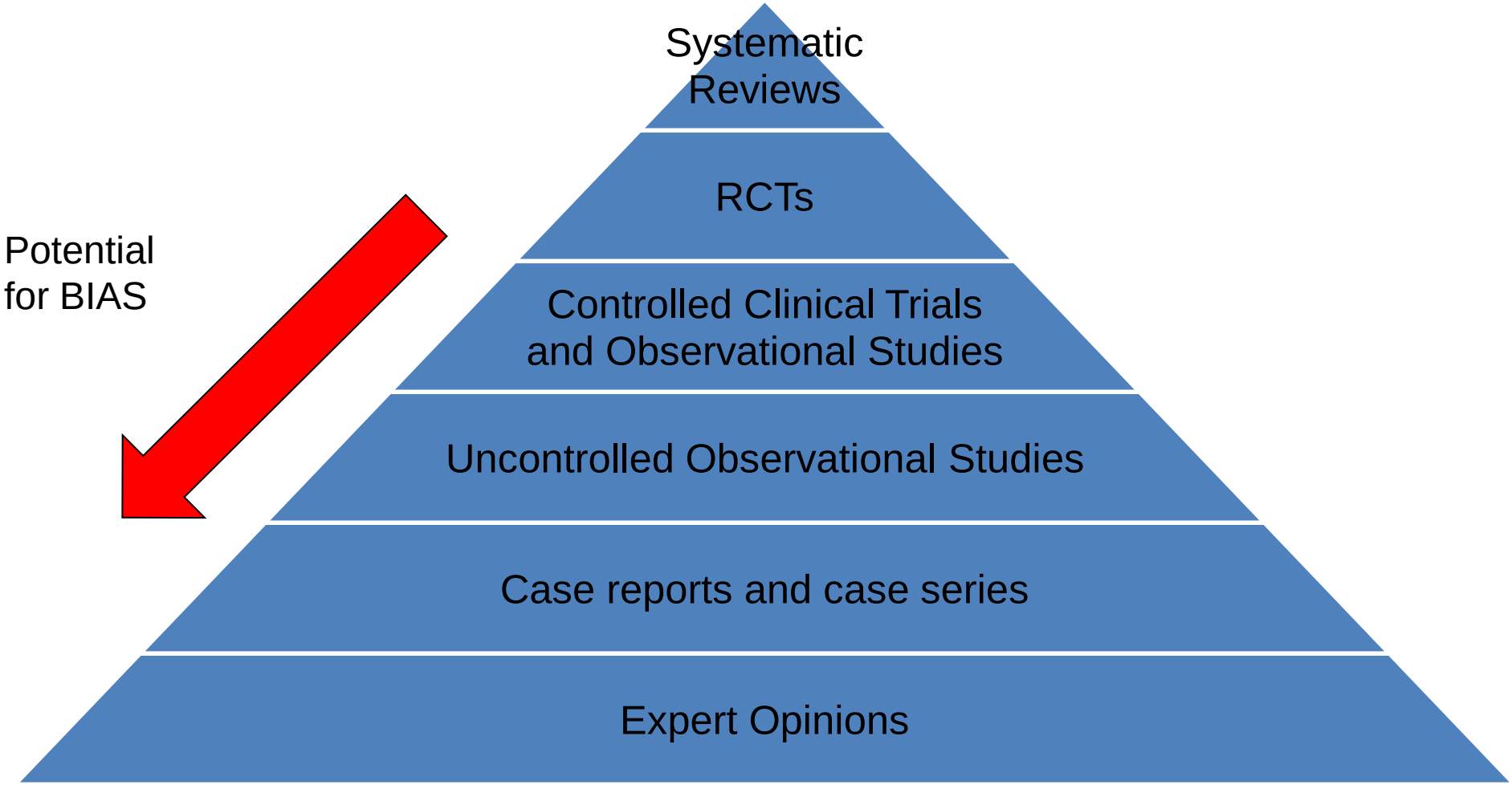
No evidence

- Doesn’t exist
- Not identified
- Not reported

Existing evidence

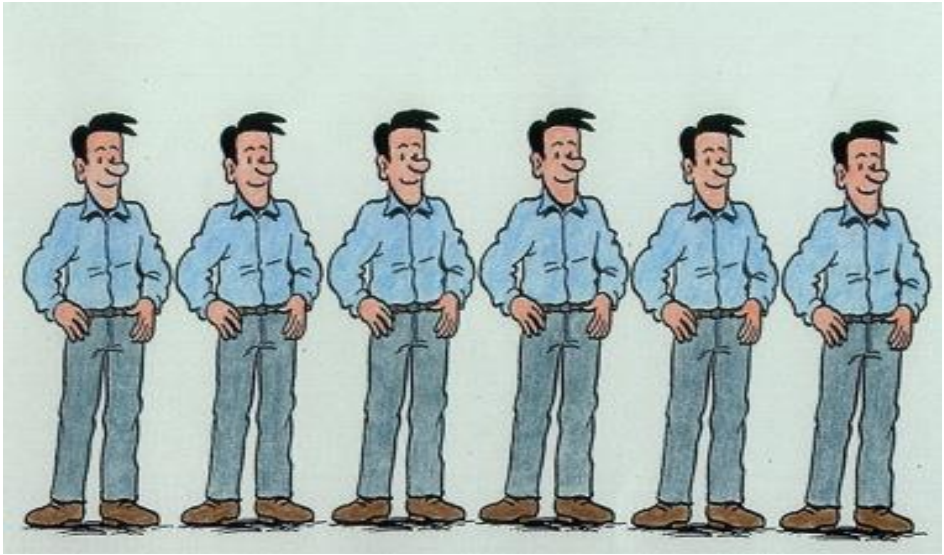
- Not robust
- Cannot be generalised (setting, patients, country etc)
- Does not answer the question
- Out of date

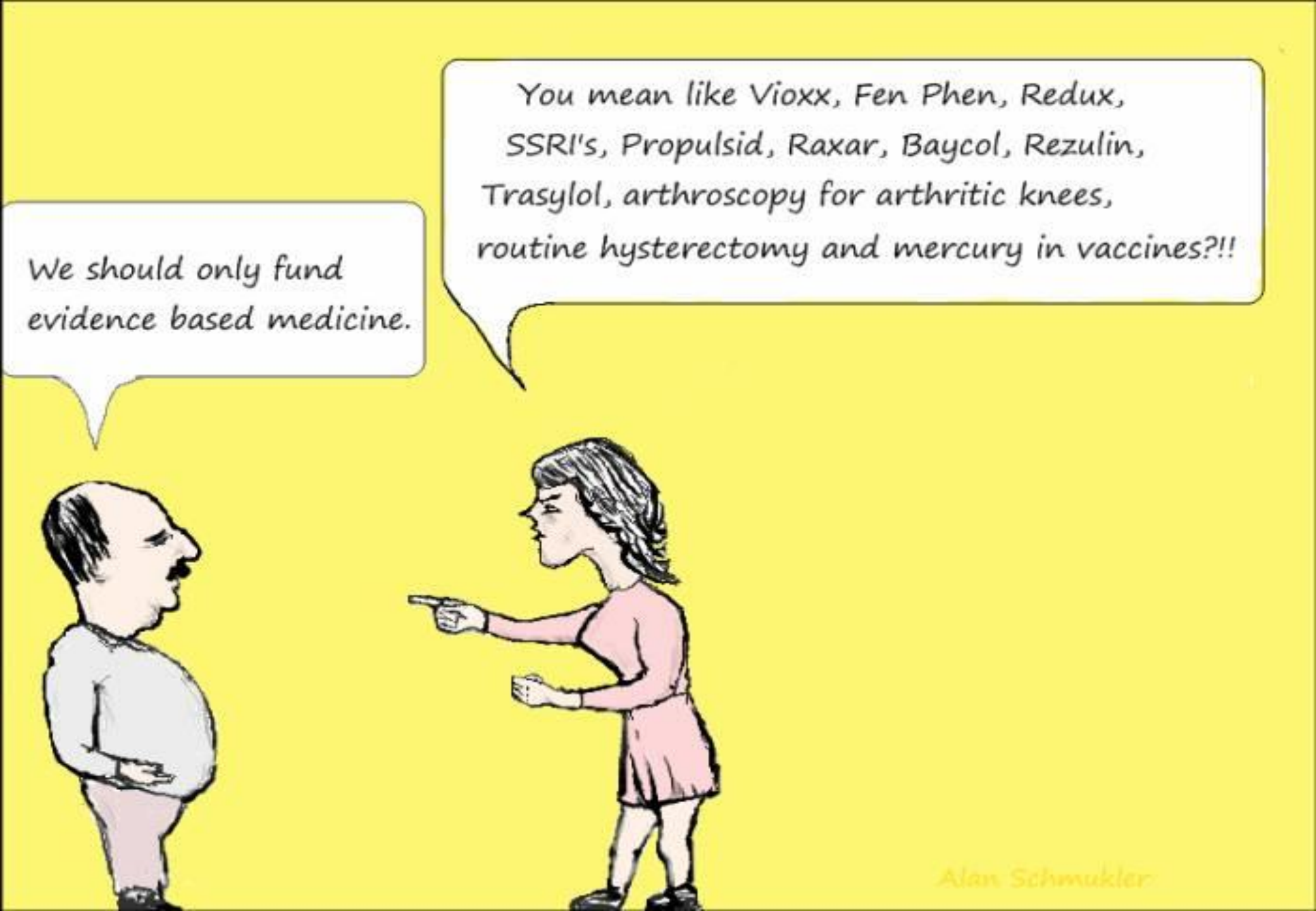
Hierarchy of Evidence



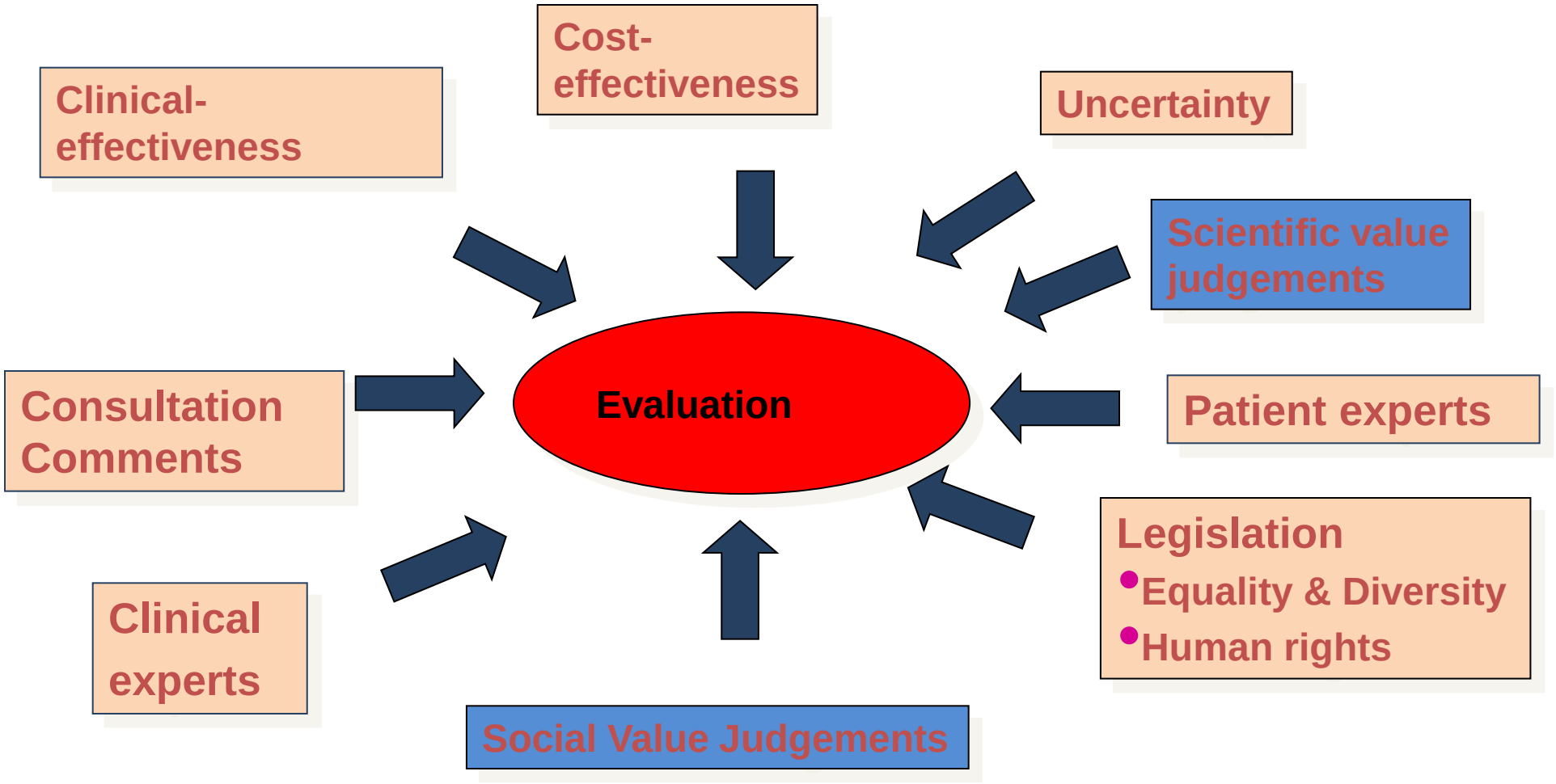
- Efficacy
- patient benefit and harm in experimental and closely monitored research studies, normally RCTs.
- Design minimises bias- high internal validity

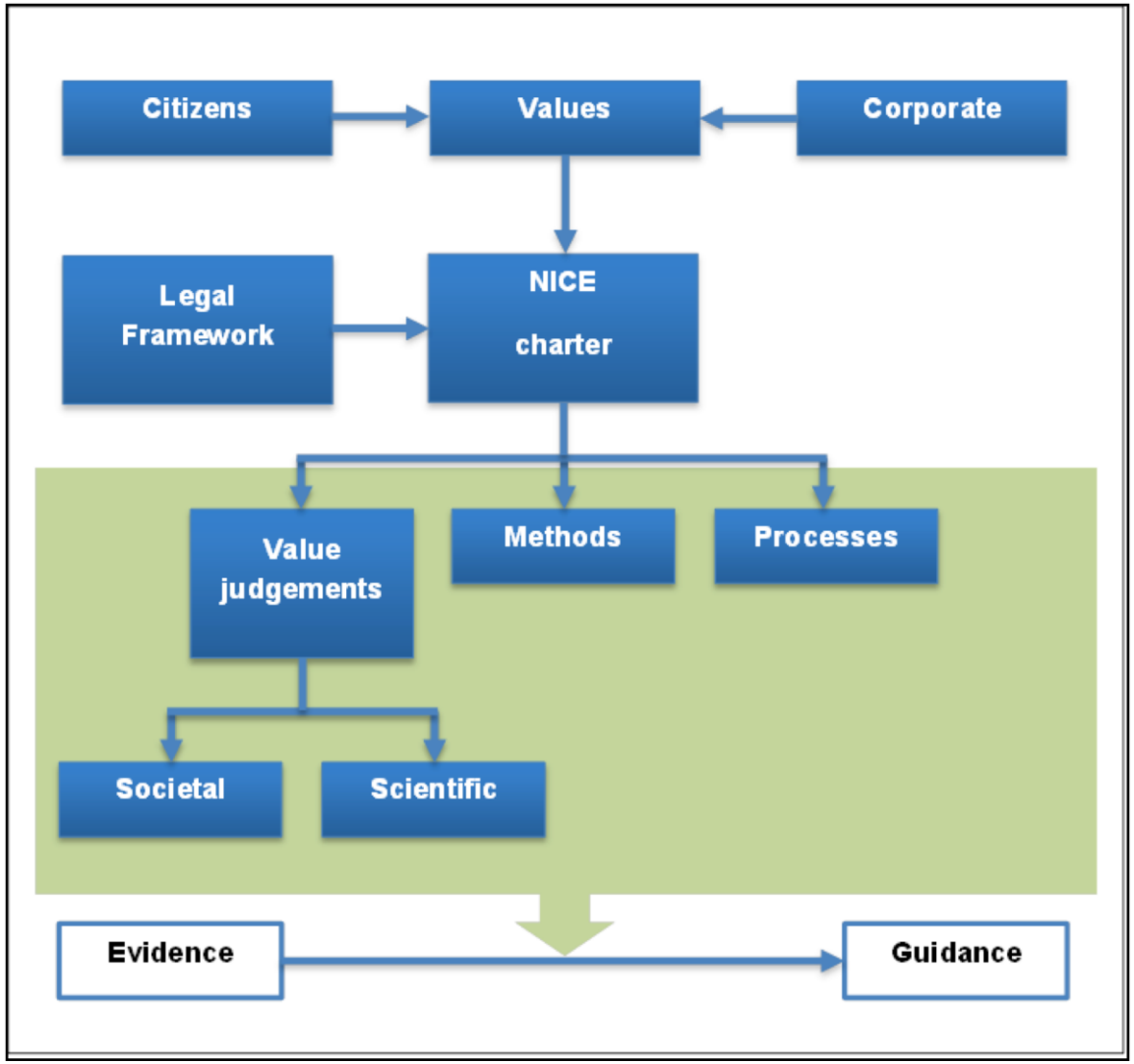
- Effectiveness
- patient benefit and harm when the technology is actually applied in everyday practice.
- *"evidence used for decision-making that is not collected in conventional randomized controlled trials (RCTs)" * ISPOR*




We should only fund evidence based medicine.
You mean like Vioxx, Fen Phen, Redux, SSRI's, Propulsid, Raxar, Baycol, Rezulin, Trasylol, arthroscopy for arthritic knees, routine hysterectomy and mercury in vaccines?!!
Alan Schmukler

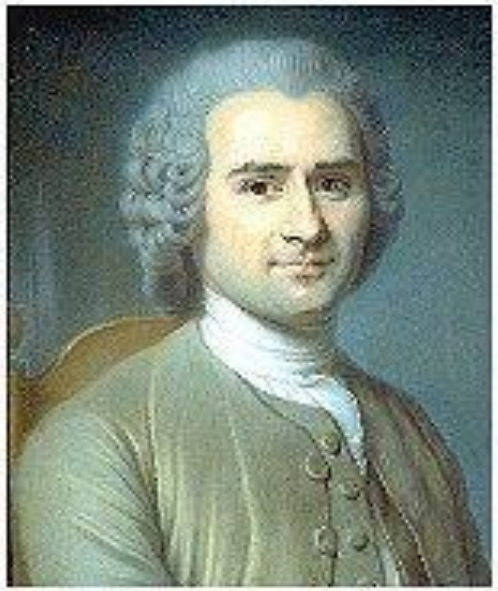
What gets taken into account?

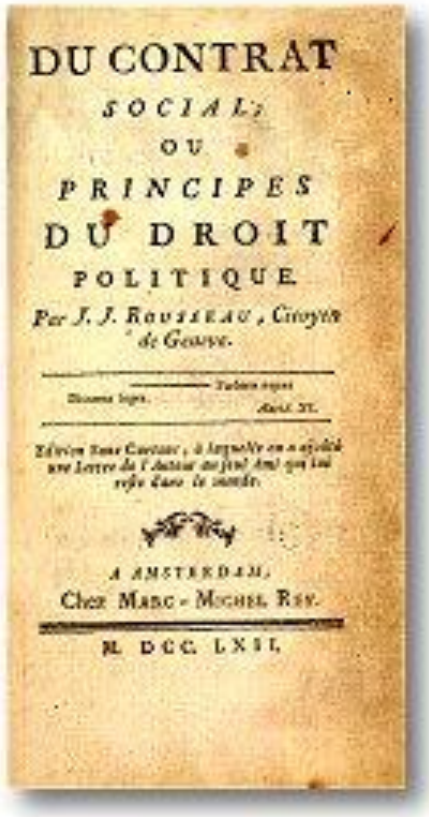



Citizens
Values
Corporate
Legal Framework
NICE charter
Value judgements
Methods
Processes
Societal
Scientific
Evidence
Guidance

The new ‘social contract’ ?

- All drugs have risk.
- Early access needs to be managed carefully.
- To ensure safety we need to evaluate.
- Health care systems are resource-limited.
- We are all tax payers and patients





Revolution or Evolution?

- Staggered approval
- Adaptive licensing
- Progressive licensing
- Conditional authorisation
- Accelerated approval
- Managed entry
- Only in research
- Coverage with evidence development
- Scientific advice
- Novel trial design
- Medicines Adaptive Pathways to Patients