



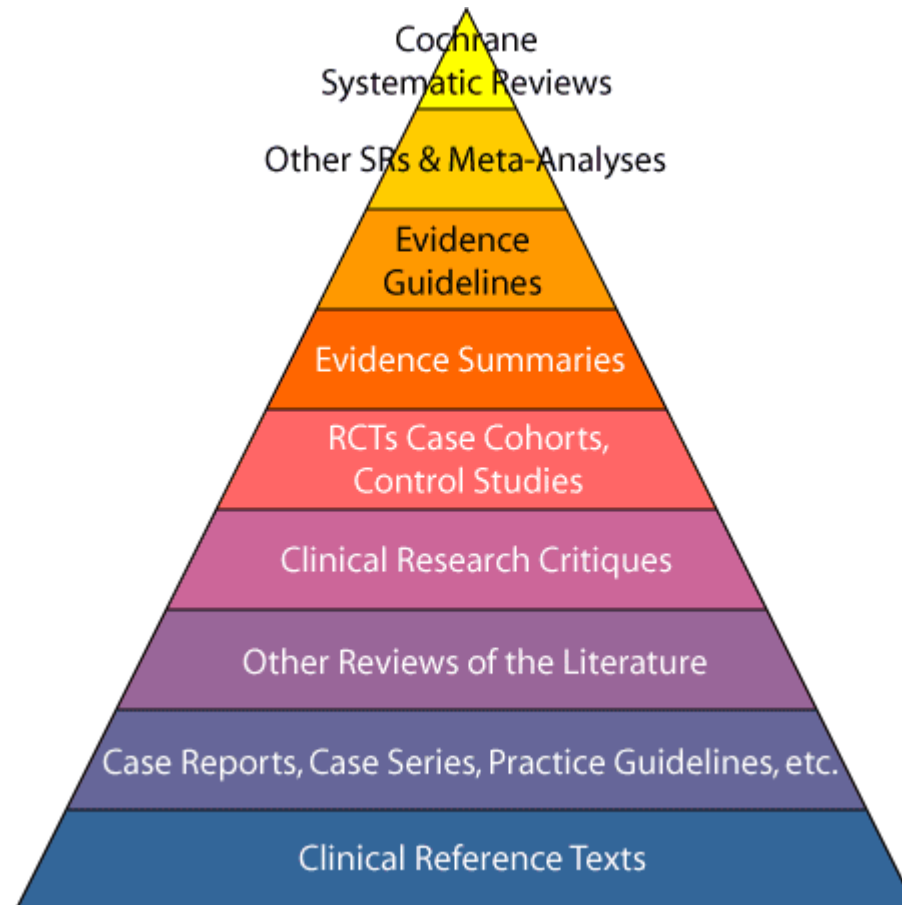
Real World Evidence for Real World Challenges

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Background

- ▶ Definitions
 - ▶ ISPOR TASK FORCE: We defined RW data as data used for decision making that are not collected in conventional randomized controlled trials (RCTs).
 - ▶ According to the European Forum “Relative Effectiveness” Working group it is, “a measure in understanding health care data collected under real life practice circumstances”; at the payer level a real-life study has been defined as “anything that is not interventional”.
- ▶ Terrible definitions of Real World Evidence.

Evidence



The RCT

- ▶ Clinical trials that involve at least one test treatment and one control treatment, concurrent enrolment and follow-up of the test- and control-treated groups, and in which the treatments to be administered are selected by a random process, such as the use of a random-numbers table. Treatment allocations using coin flips, odd-even numbers, patient social security numbers, days of the week, medical record numbers, or other such pseudo- or quasi-random processes, are not truly randomized and trials employing any of these techniques for patient assignment are designated simply Controlled Clinical Trials
- ▶ Randomized double blind placebo control (RDBPC) studies are considered the “gold standard” of epidemiologic studies.
 - ▶ Bias
 - ▶ The Counter-factual

The Real World

- ▶ Blinding not possible
- ▶ Randomisation not ethical
- ▶ Clinicians vary in education and skill
- ▶ Standard of care varies greatly by country and even locality
- ▶ Resource use important to decision making – RCTs almost never relevant
- ▶ Regimen adherence issues (is it difficult to follow)
- ▶ Guidelines
- ▶ Decision Outcomes

The Real World – Some more problems



Some Tools

- ▶ Pharmacovigilance
- ▶ Patient Matching
- ▶ Guideline Trackers
- ▶ Model External Validation (e.g. Mount Hood Challenge in Diabetes)
- ▶ EU Transparency Directive
- ▶ DRGs/HRGs
 - ▶ Try and link actual resource use to disease
- ▶ Patient Registries



The Laboratory is never the Real World



Volkswagen CEO resigns amid emissions scandal, Porsche CEO to take over



"I have the results of your PET scan and your CT scan. You are not claustrophobic."

Transparency

- ▶ Transparency Directive Says So...
- ▶ “Many Eyes”
 - ▶ Help identify “bad decisions” or mistakes in the decision making process
- ▶ Not “All Good”
 - ▶ Vaccination Adverse Events
- ▶ Not “All bad”
 - ▶ **Cancer risk in patients with rheumatoid arthritis treated with anti-tumor necrosis factor alpha therapies: does the risk change with the time since start of treatment?**¹
 - ▶ During 25,693 person-years of followup in 6,366 patients newly starting anti-TNF, 240 first cancers occurred, yielding an RR of 1.00

1. Askling J et al, *Arthritis & Rheumatism*. 2009 Nov;60(11):3180-9.

Conclusion

- ▶ It's ALL about the analytics
 - ▶ **BIG DATA IN HEALTH CARE** The Fourth International Conference of the Pharmacoeconomics Section of the Pharmaceutical Association of Serbia Belgrade, 15th and 16th of May 2014 MSc pharm. Tanja Novakovic
 - ▶ Our ability to ANALYSE Real World Evidence is only just catching up with our ability to COLLECT Real World Evidence.
- ▶ Need Better definitions of Real World Evidence and clearer guidance on using it.