

# Beyond Efficacy: Hard Outcomes in Treatment Studies

Guy Goodwin, University of Oxford,  
UK

# Is there a problem?

## Efficacy and safety established

Well validated scales  
Randomized clinical trial  
Placebo  
Short & long term data

## Effectiveness, safety (and reimbursement)

Hard outcomes  
(economic data)  
Non-random  
No placebo  
Key is long term

# Randomized clinical trials

## Pro

Highest level of evidence  
Low risk for systematic bias  
Demonstrate “*efficacy*”

## Contra

Narrow inclusion and exclusion criteria limits generalisability  
Difficult to demonstrate effect of old drugs  
“Enriched design” benefits the study drug  
Do not show “*effectiveness*” in the real world  
Expensive  
Long-term trials might be unethical

# The agenda for this session

	Within-subject comparison as a tool to explore effectiveness in register studies	M Landén
	Discussion	
	Relapse prevention studies: What happens after drop out?	S Leucht
	Discussion	
	Industry Perspective: Risk characterization utilizing external data sources: A methodological consideration for clinical trials with high subject drop-out	J Geier
	Discussion	
	Regulatory Comment/Panel Audience Discussion	M Tome Speakers
	Session 1 Adjourns	
	Break	