

Overcoming the challenges of randomised trials of musculoskeletal pain

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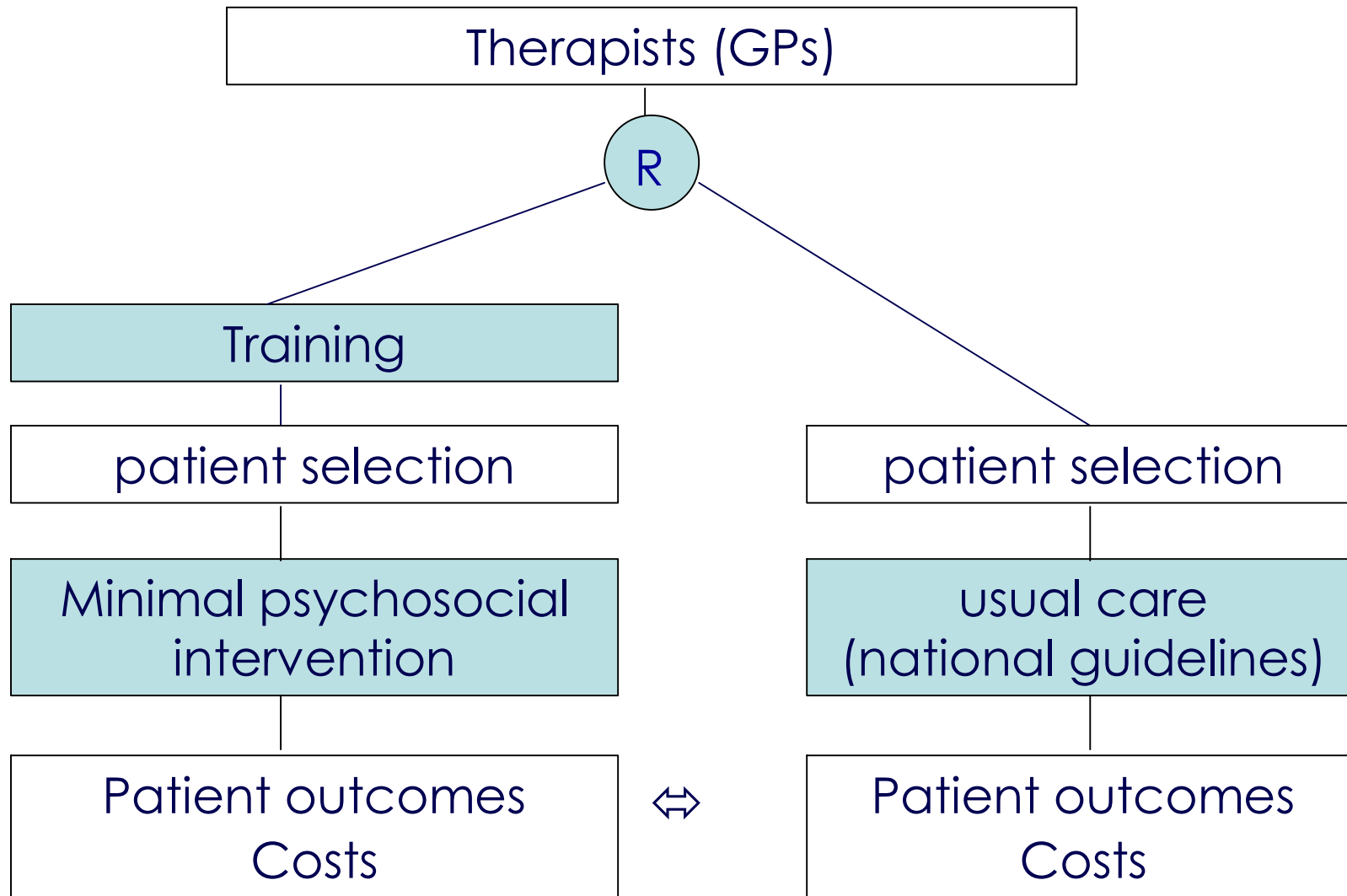
Introduction

- RCTs – appropriate method to evaluate PT interventions (high internal validity)
- Designing trials that are relevant to clinical practice is challenging (how to optimise external validity)
- Two issues:
 - therapist effects (competencies, skills)
 - beliefs, expectations, preferences
- How can we address these issues in RCTs?

I. Therapist effects - risk of contamination

- Assumption of RCTs: prognostic similarity at baseline
 - patients: e.g. duration and severity of musculoskeletal pain
 - therapists: skills, competencies, experience
- Complex interventions
 - therapists may need training to provide intervention
 - therapists may have particular interest in one intervention
- Randomisation solves problems regarding baseline similarity
 - randomisation at patient level: therapists deliver more than one intervention: risk of contamination?
 - randomisation at therapist level: cluster-randomisation

Cluster-RCT: example



Cluster-randomised controlled trials

- Advantages
 - no risk of contamination
 - similarity between intervention groups regarding experience, skills, beliefs of therapists
- Challenges
 - baseline similarity of patients?
 - selection of patients preferably carried out by independent researcher team (not therapists themselves)
 - ethical issues
 - effects of clustering within therapists

Therapist effects - influence on outcome

- Within intervention groups
 - variation in enthusiasm, skills & competencies of therapists
 - variation in beliefs, attitudes, and expectations of therapist
 - therapist-patient interaction
- Outcome may vary across therapists
 - effects of clustering of patients within therapists / practices
 - explore therapist effects / adjust for in analysis
- Process evaluation
 - measure skills, attitudes, beliefs
 - measure content of therapy

II. Expectations and preferences

- Positive beliefs and expectations (patients and therapists) may enhance non-specific effects
- Important in pragmatic trials (no blinding of patients, subjective outcomes)
- When allocated to the non-preferred treatment:
 - resentful demoralisation
 - drop-out, non-compliance
 - relatively poor results

Example 1: shoulder pain - NL



	Success rate at 6 weeks	
	Injections (n=52)	Physiotherapy (n=56)
Overall	77%	46%
No preference	80%	47%
Randomised to preferred Tx	85%	53%
Not randomised to preferred Tx	64%	50%

- Treatment preferences seem to influence outcome, but more for injections than for physiotherapy
- Small numbers ...

Example 2: shoulder pain - UK



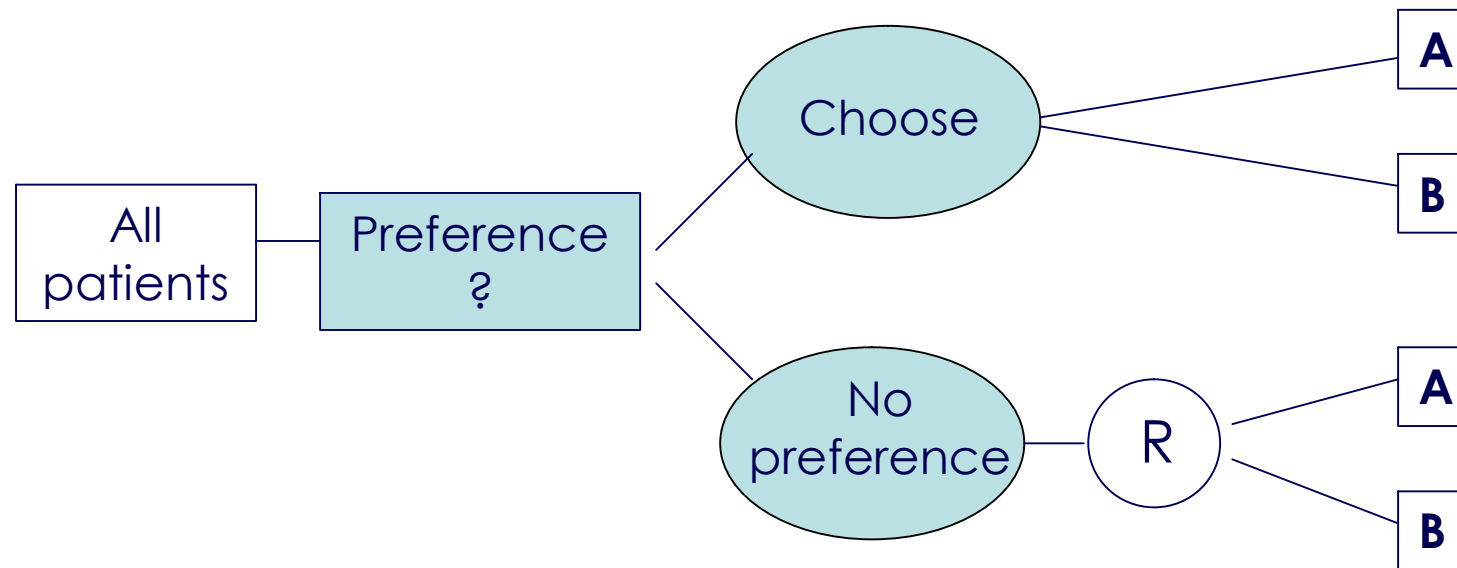
- Having a preference positively influences outcome, regardless if preferences are met
- Good outcomes lead to stronger preferences

	Good functional outcome 6 months	
	Injections (n=104)	Physiotherapy (n=103)
No preference	42%	54%
Preference	59%	64%

Challenges

- Patients with strong preferences often do not consent to randomisation
 - influence of strong preferences cannot be studied
 - difficult to generalise results of RCTs
- Alternative designs
 - complex
 - require large numbers
 - do not completely solve the problem

Alternative designs - example



Comprehensive cohort design (Ricker / Wennberg)

Patient preference design (Brewin & Bradley)

Summary & conclusions

- Variation in skills & competencies of therapists may influence outcome (therapist effects)
- Patient preferences & expectations may influence outcome
- Influences of these issues can be addressed within the framework of RCTs
 - use alternative designs (e.g. cluster-randomisation)
 - measure all relevant factors during the trial
 - explore potential influences (process evaluation)

Thank you

