South East Wales Trials Unit

Uned Ymchwil De-ddwyrain Cymru

sewtu

CARDIFF UNIVERSITY

PRIFYSGOL CAERDYDD

"Should I even bother attending?"

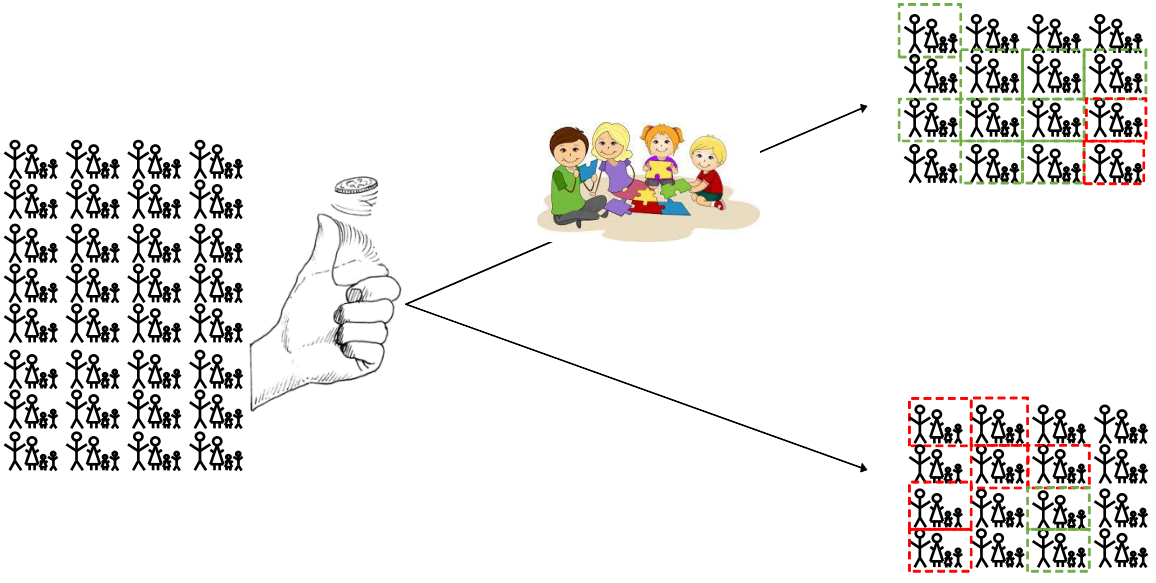
Determining Maximal Intervention Benefit from Public Health Trials using Randomisation-Based Efficacy Estimators

David Gillespie, Laurence Moore, Jeremy Segrott, Sharon Simpson, Mark Kelson, Paul Willner, Kerenza Hood

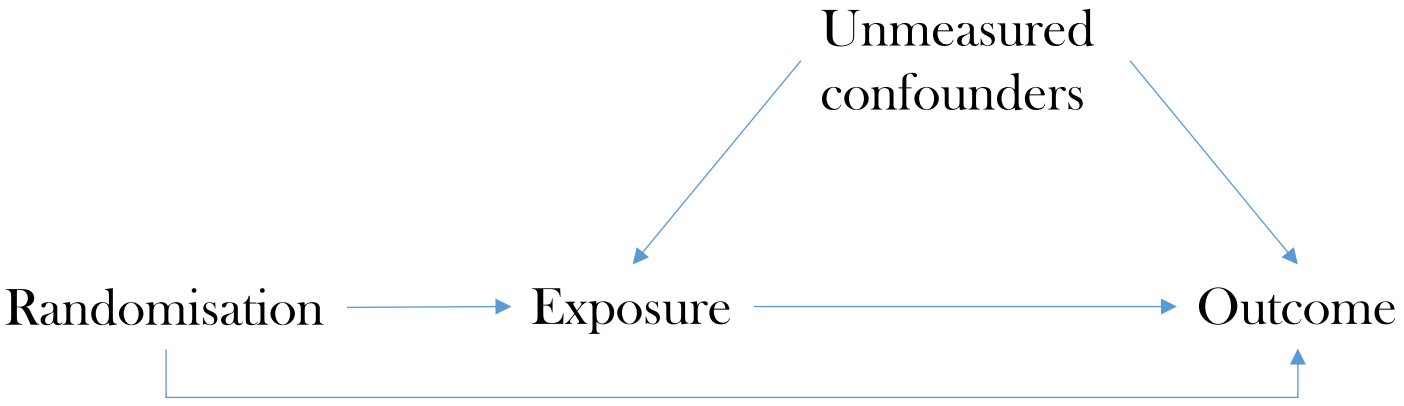
SCT Annual Meeting - Monday May 18th 2015 - Arlington, Virginia

Motivation

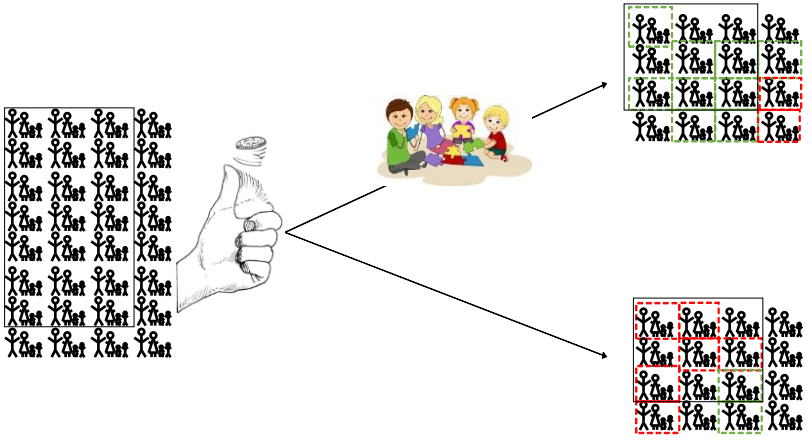
- Why evaluate interventions using RCTs?
- Intention to treat analysis
- Compliance with public health interventions
- Traditional efficacy analysis



Randomisation-based efficacy



- Selection bias
- Potential outcomes
- Randomisation as an instrument
- Binary or quantitative exposure



Study 1: Project SFP Cymru

PICO description of Project SFP Cymru	
Participants	Families with adolescents aged 10 to 14 years
Intervention	Strengthening Families Programme
Control	Usual care / Locally available services
Outcome	Adolescent alcohol use in the previous 30 days at 24 months post-randomisation

- Intervention receipt defined as attending five or more sessions without missing more than one in a row
- Total number of weeks attended also used

Study 1: Project SFP Cymru

- 66% of adolescents "received" the intervention
  - 37% attended all 7 sessions
  - 16% attended 0 sessions

Analysis	Adjusted odds ratio for alcohol use at 24 months*	Lower 95% CI	Upper 95% CI	p-value
ITT	1.12	0.72	1.73	0.625
CACE (binary)	1.21	0.79	1.86	0.384
SMM (per week)	1.03	0.97	1.09	0.382
SMM (per 7 weeks)	1.20	0.79	1.83	

*SFP compared to control. Adjusted for average age of adolescents within the family, recruitment area, level of challenge exhibited by the family.

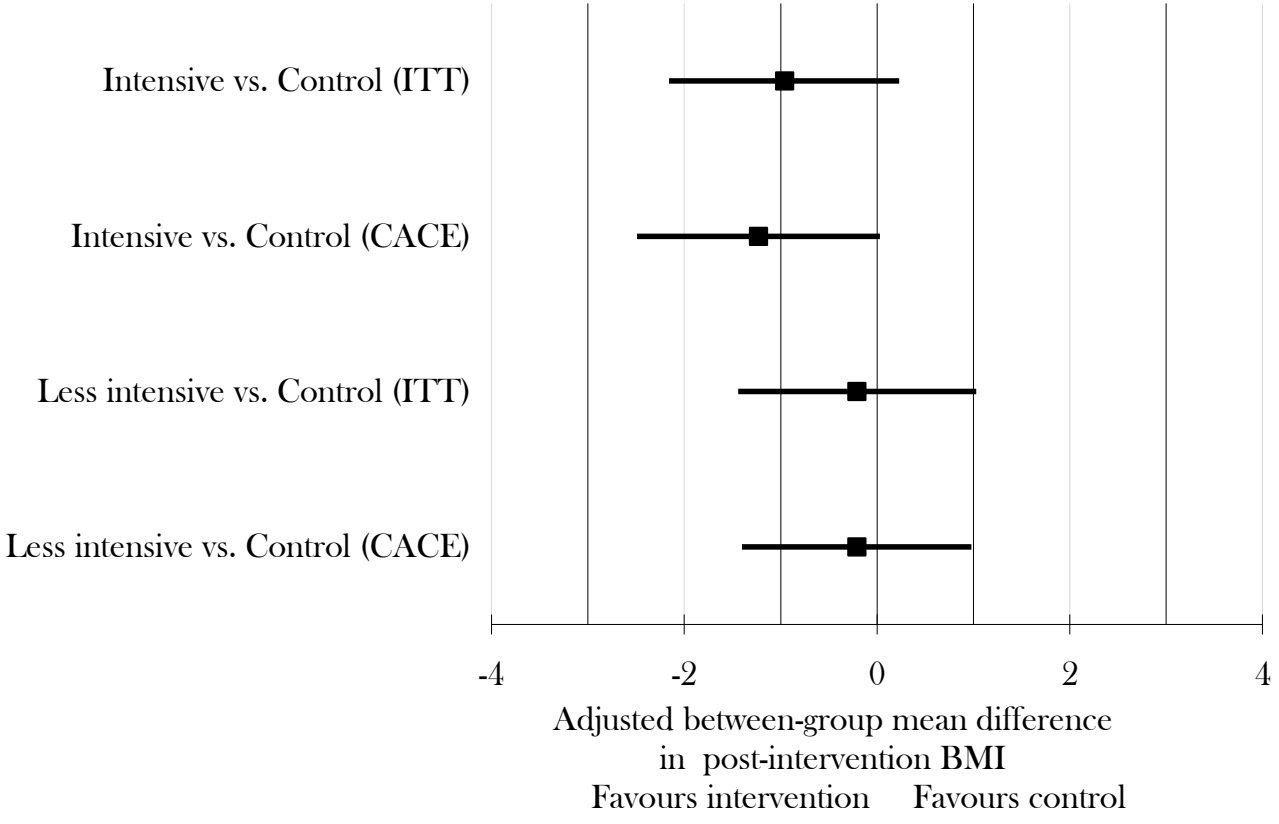
Study 2: The WILMA trial

PICO description of WILMA	
Participants	Overweight adults who had lost at least 5% of their bodyweight in previous year
Intervention(s)	Individually-tailored motivational interviewing
Control	Leaflet on healthy lifestyle
Outcome	BMI (kg/m^2) at 12 months post-randomisation

- Intervention receipt defined as attending
  - 5/6 face-to-face sessions (intensive arm)
  - 2/2 face-to-face sessions (less intensive arm)

Study 2: The WILMA trial

- **Intensive arm: 83% ; Less intensive arm: 91%**
- Potential clustering by therapist (individually RCT):
  - Outcome ICC = 0, **Compliance ICC = 0.057**, small cluster sizes (**average = 3**)
  - Unlikely to bias variance/standard error estimation (Jo et al., Jo et al.)
- Three arm trial (Cheng J, Long Q)



Study 3: The Anger Management trial

PICO description of Anger Management	
Participants	Adults with mild to moderate learning disabilities who had problems managing their anger
Intervention	12-week group-based cognitive behavioural therapy
Control	Waiting list care as usual
Outcome	Provocation Index at 10 months post-randomisation

- Intervention receipt defined as attending 8/12 sessions

Study 3: The Anger Management trial

- 75% of participants attended 8/12 sessions
  - 30% attended all 12 sessions
  - 15% attended 0 sessions
- Potential clustering by centres (cluster RCT):
  - Outcome ICC = 0.2, compliance ICC = 0.4, but cluster sizes again small (average = 6)
- Analysis adjusts for clustered nature of outcome, but not compliance

Analysis	Adjusted mean difference in PI at 10 months*	Lower 95% CI	Upper 95% CI	p-value
ITT	-2.8	-7.4	1.7	0.210
CACE	-3.3	-7.9	1.4	0.165

*Intervention minus control. Adjusted for baseline PI and clustering of participants within centres.

Summary

- Added value of RBEEs
- Answer question of value to participants
  - Potentially to policy makers too
- Linearity versus exclusion restriction
- Clustering
- Missing data

- For more info: GillespieD1@Cardiff.ac.uk

DaveGuk87

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