Why we should do more Pragmatic Trials and how PRECIS-2 could help

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Aims for today

- Why are Pragmatic trials & PRECIS-2 important?
- PRECIS-2 tool
- Testing PRECIS-2 at the PCTU
- Using PRECIS-2 in 2 pilot studies
- Key design challenges discussed with users
- Conclusions

EXPLANATORY AND PRAGMATIC ATTITUDES IN THERAPEUTICAL TRIALS

DANIEL SCHWARTZ and JOSEPH LELLOUCH

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(*Received* 6 *January* 1967; *in revised form* 24 *March* 1967)

It is the thesis of this paper that most trials are inadequately formulated. Their inadequacy is basic, in that trials may be aimed at the solution of one or other radically different kinds of problems.

Most trials done hitherto have adopted the explanatory approach without question; the pragmatic approach would often have been more justifiable.

J. chron. Dis. 1967, Vol. 20, pp. 637–648. Pergamon Press Ltd.

Why is it important?

- **Pragmatic trials** – Trials that test if an intervention will work in ***usual*** conditions
- **Explanatory trials** – Trials that test if an intervention will work in highly controlled, ***ideal*** circumstances
- **Continuum** *not* Dichotomy

Explanatory and pragmatic attitudes in therapeutical trials (1967).

Schwartz D, Lellouch J. Republished in J Clin Epidemiol. 2009;62(5):499-505

71% of RCTs do not represent real world patients

Kennedy-Martin *et al. Trials* (2015) 16:495
DOI 10.1186/s13063-015-1023-4

TRIALS

REVIEW Open Access

CrossMark

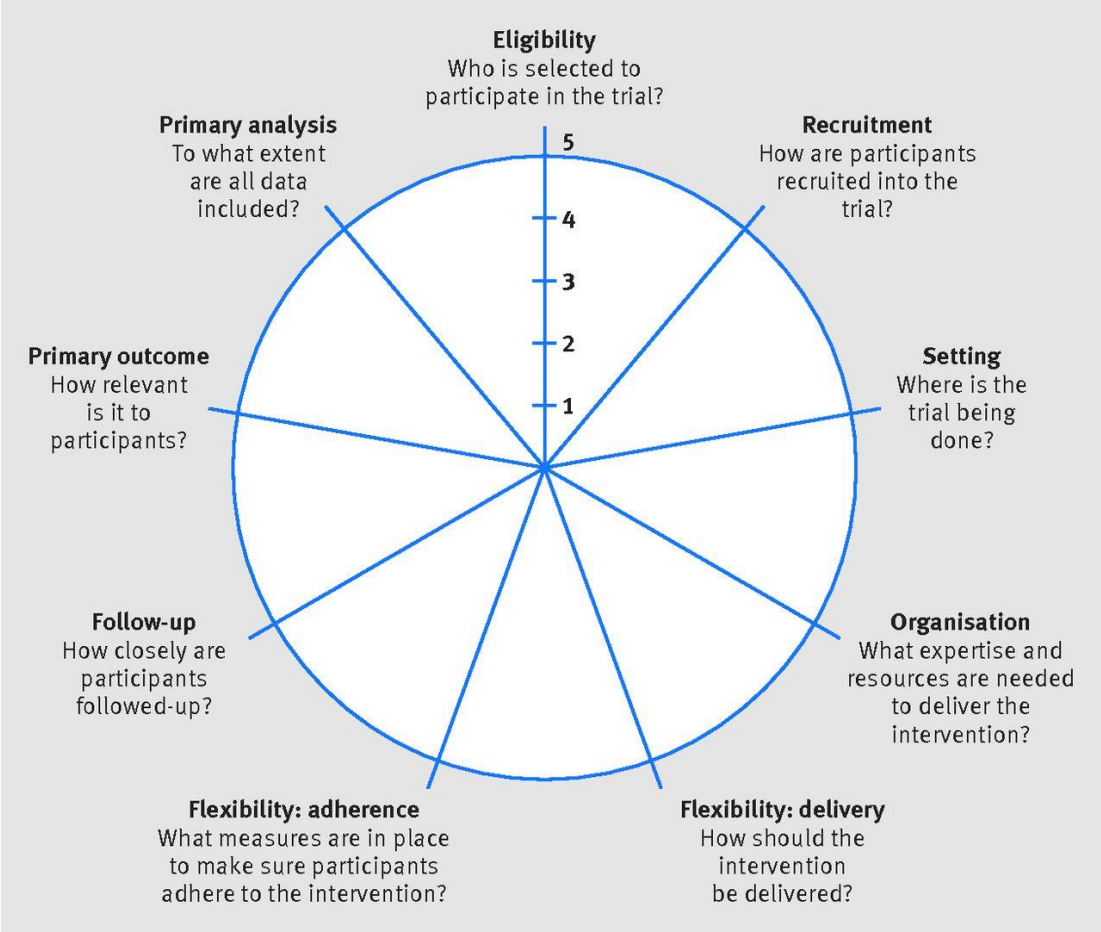
A literature review on the representativeness of randomized controlled trial samples and implications for the external validity of trial results

Tessa Kennedy-Martin[1*], Sarah Curtis[2], Douglas Faries[2], Susan Robinson[1] and Joseph Johnston[2]

Abstract

Randomized controlled trials (RCTs) are conducted under idealized and rigorously controlled conditions that may compromise their external validity. A literature review was conducted of published English language articles that reported the findings of studies assessing external validity by a comparison of the patient sample included in RCTs reporting on pharmaceutical interventions with patients from everyday clinical practice. The review focused on

PRECIS-2 wheel



thebmj | BMJ 2015;350:h2147 | doi: 10.1136/bmj.h2147

PRECIS-2 key feature

Improve ***applicability*** of trial results

HELP trialists think about ***consequences*** of ***design decisions***

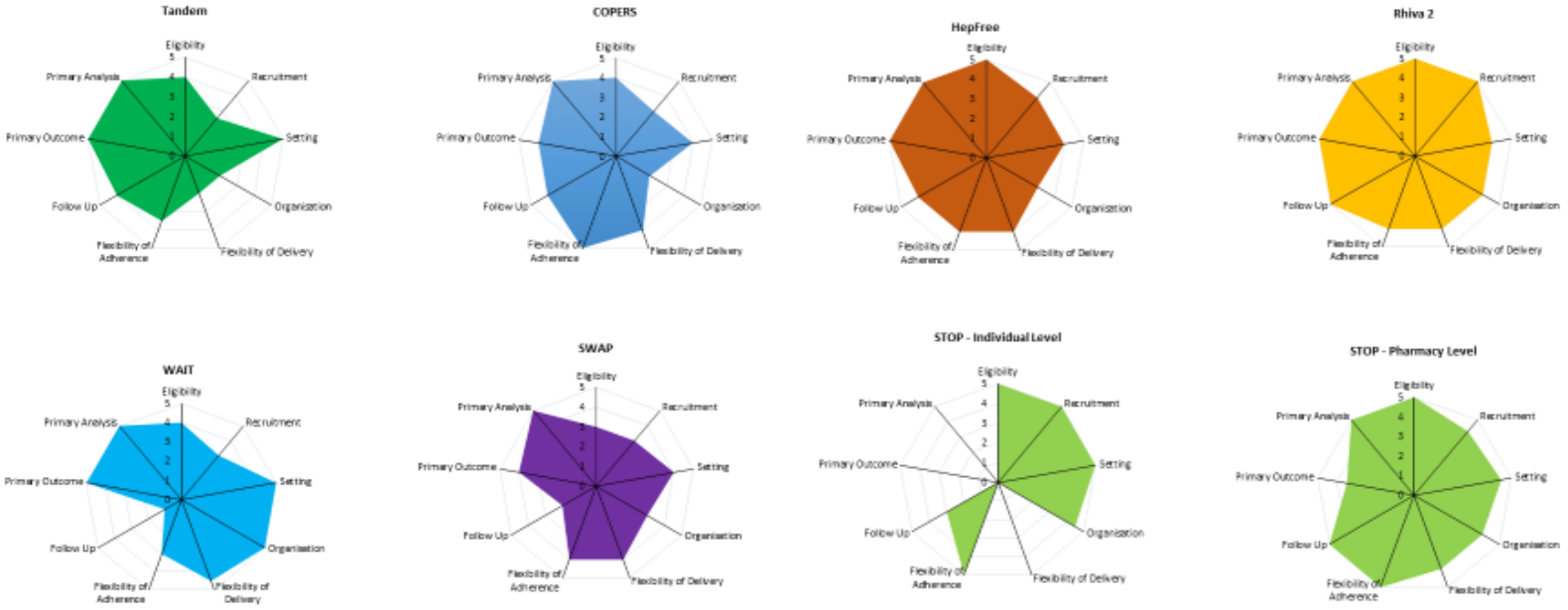
So trialists consider "***how different is the trial…. to usual care***"

Organisation domain in PRECIS-2

- How **different are the resources, provider expertise and the organisation of care delivery** in the intervention arm of the trial and those available in **usual care**?

- For example, score **5** for very pragmatic choice with identical organisation to usual care;
- score **1** for a very explanatory approaches if the trial increases staff levels, gives additional training, requires more than usual experience or certification and increase resources.

Pragmatic Clinical Trials Unit trials



Forbes G. Understanding the applicability of results from primary care trials: lessons learned from applying PRECIS-2 J Clin Epi 2017G

Using PRECIS-2 to discuss the design of a feasibility study: SiP

A Swallowing intervention Package (SiP)

Methods

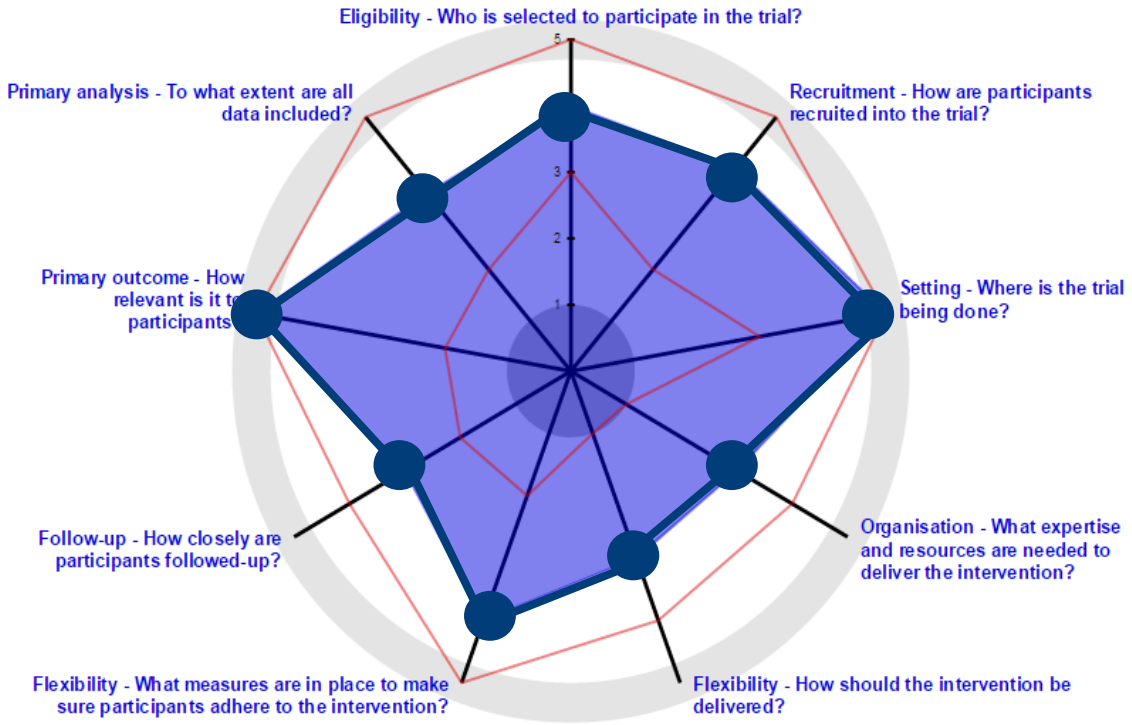
- 14 trial team members
- 11 PRECIS-2 wheels independently created pre-meeting
- Meeting to discuss results

Results

- Greater consensus following meeting
- Useful tool to frame the conversation around discussing trial design
- Useful tool to assess pilot study and future RCT
- Fun

Wells M. Pilot and Feasibility Studies 2016**2**:40

SiP feasibility study



Useful to assess feasibility study and future RCT

Process Evaluation in Pilot Study

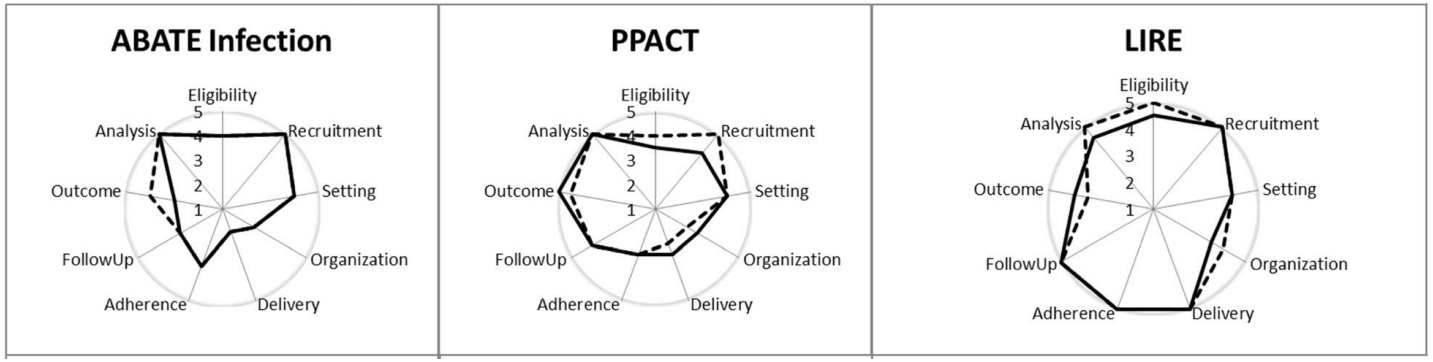
- Ease and suitability of the PRECIS-2 tool to be used as an assessment tool during the process and implementation evaluation.
- Pre-intervention
- During intervention
- Post-intervention

Pragmatic trial assessment

RESEARCH Open Access

Use of PRECIS ratings in the National Institutes of Health (NIH) Health Care Systems Research Collaboratory

Karin E. Johnson[1†], Gila Neta[2*†], Laura M. Dember[3], Gloria D. Coronado[4], Jerry Suls[2], David A. Chambers[2], Sean Rundell[5], David H. Smith[4], Benmei Liu[2], Stephen Taplin[2], Catherine M. Stoney[6], Margaret M. Farrell[2] and Russell E. Glasgow[7]



Few challenges of Pragmatic trials

- Usual care including outside NHS
- Blinding
- Recruitment and informed consent
- Working with collaborators
- Organisation limitations

PRECIS-2 website

Top 10 Countries

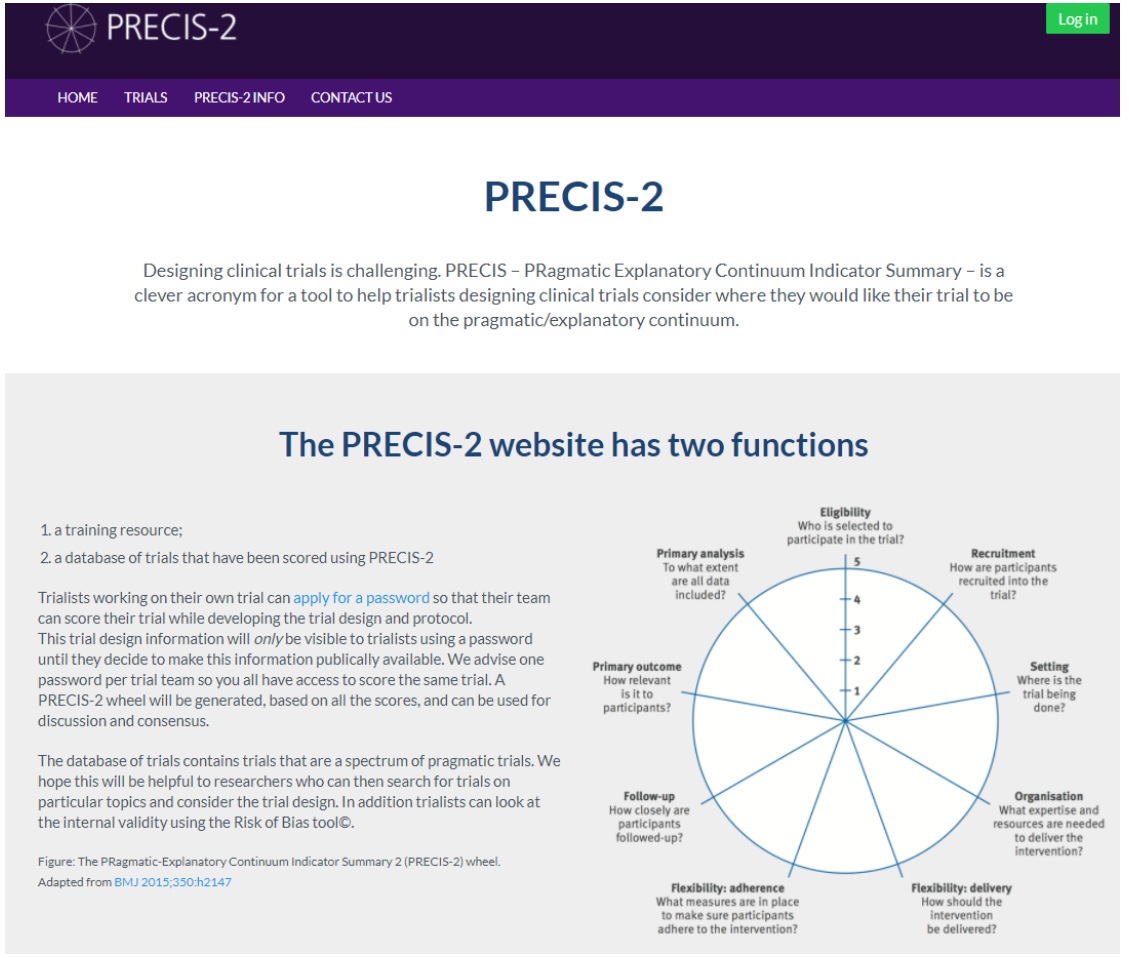
1. United States
2. United Kingdom
3. Canada
4. Spain
5. Australia
6. Japan
7. Norway
8. Netherlands
9. New Zealand
10. Austria

Registered users

363 entries

Number of wheels

309 entries



www.PRECIS-2.org

Summary & Conclusions

- PRAGMATIC trials test interventions in routine practice
- PRECIS-2: An efficient way to DESIGN more pragmatic trials that are more likely to meet the needs of patients, health care professionals
- Increases TRANSPARENCY in design decision making and helps guide the conversation about design from pilot to full RCTs.

Useful References and Websites

PRECIS-2 Website: www.PRECIS-2.org

Users' Guide to Pragmatic Trials http://crispebooks.org/PragmaticTrialsEbook

NIH Collaboratory Rethinking Clinical Trials http://www.rethinkingclinicaltrials.org/

Implementation Science Website: http://dccps.cancer.gov/is/

Loudon K. Treweek S, Sullivan P, Donnan P, Thorpe KE, Zwarenstein M. The PRECIS-2 tool: Designing trials that are fit for purpose. *BMJ* 2015;350:h2147.

Johnson KE et al, Use of PRECIS ratings in the National Institutes of Health (NIH) Health Care Systems Research Collaboratory. *Trials* 2016; 17:32

Forbes G, Loudon K et al. Understanding the applicability of results from primary care trials: lessons learned from applying PRECIS-2 J Clin Epi 2017

Zwarenstein M, Loudon K... Understanding pragmatism and PRECIS-2 J Clin Epi 2017

To be *pragmatic* or not to be, that is the question…

"A pragmatic approach to pragmatism would be to adopt the features of pragmatic trials whenever feasible and sensible and when such features do not compromise trial quality and the ability to answer the clinical question of interest."

Ford & Norrie Pragmatic Trials. NEJM **2016;375:454-63.**

Thank you for listening!

Any questions?

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