

**Statistical, Clinical, Payer and
Regulatory Perspectives on
Dimensions that Define the
Spectrum of Efficacy/
Explanatory and Effectiveness /
Pragmatic Trials**

Co-chairs

Larry Alphs

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Introduction

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The session title says it all: goals of the session - I

- Address design requirements
 - Ginger Haynes
- Introduce statistical principles
 - Doug Faries
- Show case examples
 - Ravi Anand
 - Joanne Severe
 - Larry Alphs

Goals of the session - II

- Place CNS field in context
 - Clive Adams
- Address payer needs
 - Rob Epstein
- Provide regulatory perspective
 - Ni Khin – US
 - Geoff McColl – Australia
- Summarize
 - Larry Alphs

Terminology

- Why explanatory – pragmatic
 - Efficacy and effectiveness share a common dictionary definition
- Why dimensions rather than categories
 - Most studies fall along a continuum rather than falling into categories
 - Dimensions provide potentially meaningful analytic metric

Pragmatic- Explanatory Dimensions PRECIS

- **P**R – PRagmatic
- **E** – Explanatory
- **C** – Continuum
- **I** – Indicator
- **S** – Summary

Thorpe et al *J Clin Epidemiology* 2009

10 PRECIS Dimensions

- Participant eligibility criteria
- Experimental intervention flexibility
- Experimental intervention practitioner expertise
- Comparison intervention flexibility
- Comparison intervention practitioner expertise
- Follow-up intensity
- Primary outcome measure
- Participant adherence to intervention
- Practitioner adherence to protocol
- Analysis of primary outcome

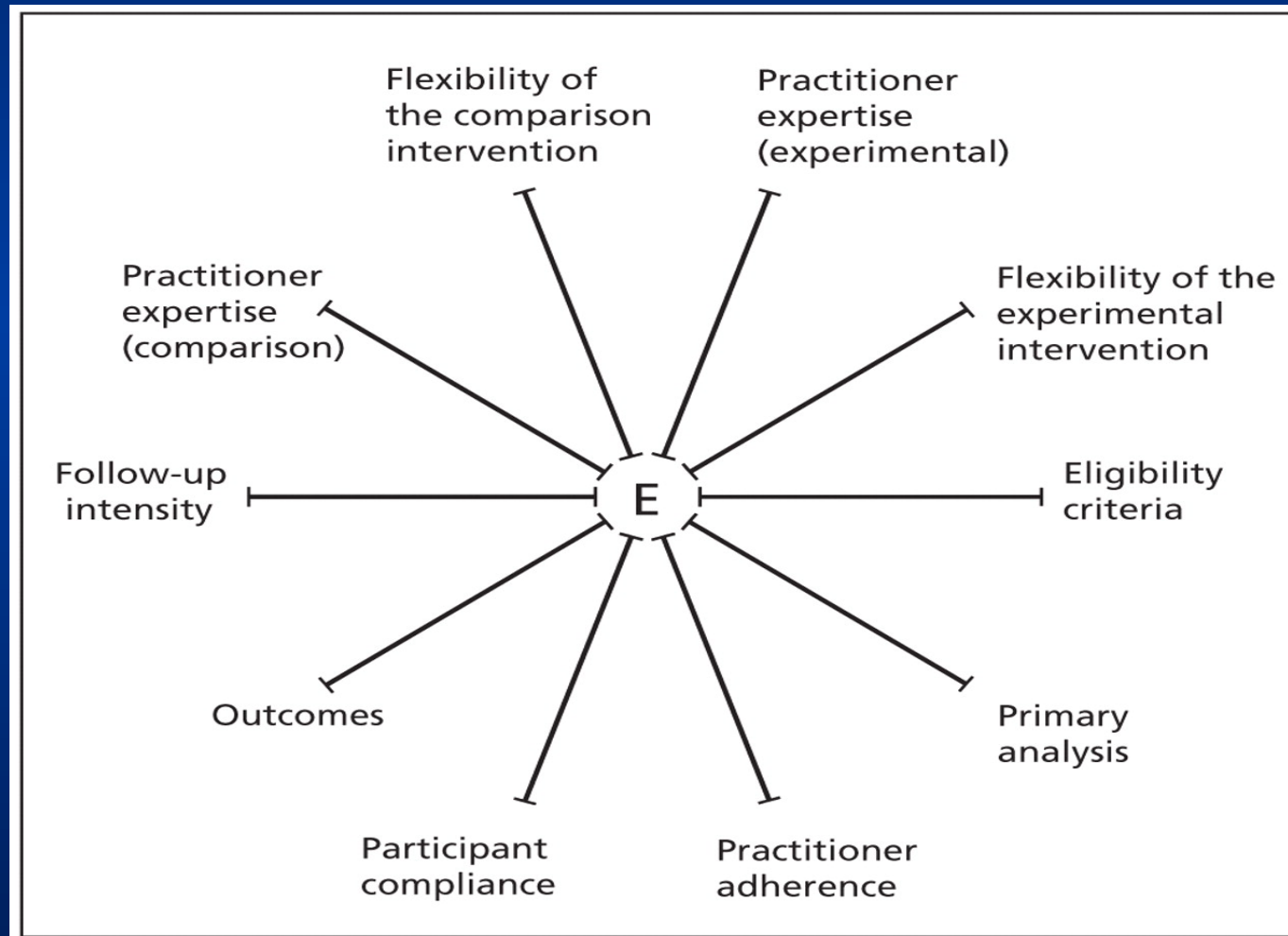
Pragmascope

A rating scale for PRECIS

- Each dimension rated on a 6 point scale
- Scale points defined for each dimension
- 0 = fully explanatory
- 5 = fully pragmatic
- Results plotted on the PRECIS wheel

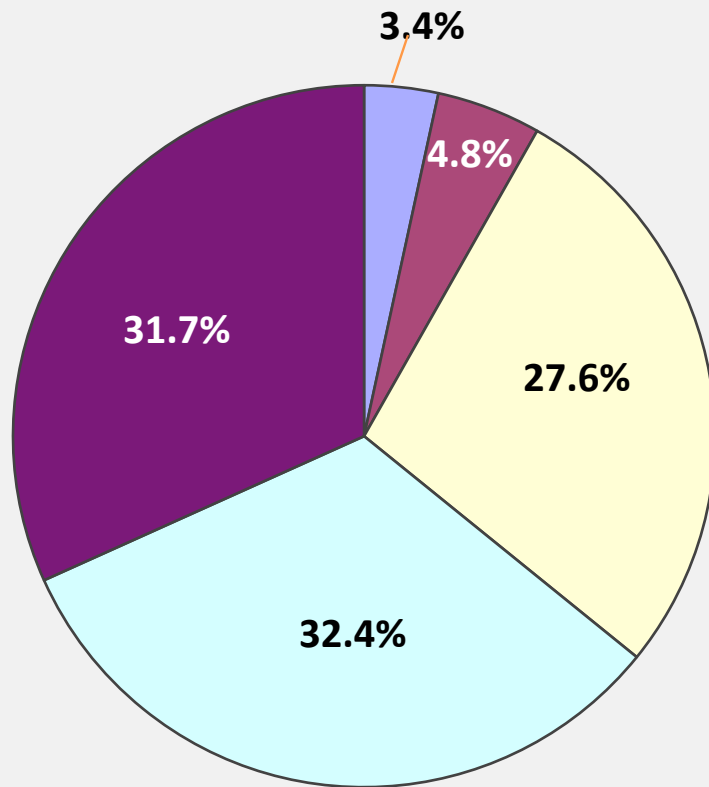
Tosh, Soares-Weiser , **Adams** *Dialogues in Clinical Neuroscience* 2011

The PRECIS wheel



What experience have you had with pragmatic (effectiveness) clinical trials (e.g., design, implementation, peer review, etc)?

N = 145/1576



- a. None
- b. Limited awareness
- c. Aware but no practical experience
- d. Limited practical experience
- e. Extensive practical experience

On To the Session
