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Eliciting Patient Perspective in Patient-Centered Outcomes Research: A Meta-Narrative Review and Synthesis of Evidence

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Outline

- Background
- Research question
- Methods
- Results
- Recommendations
- Patient feedback
- Limitations

Background

- PCOR requires meaningful patient engagement
- Methods to engage patients are unclear

Mockford at al. Int J Qual Health Care. Feb 2012;24(1):28-38.

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Research question

- Who are the relevant patients for engagement?
- How to identify and recruit them?
- How can they engage?
- How can their engagement result in changes in research design, conduct, analysis and dissemination?
- Ultimate goal: to provide recommendations

Methods

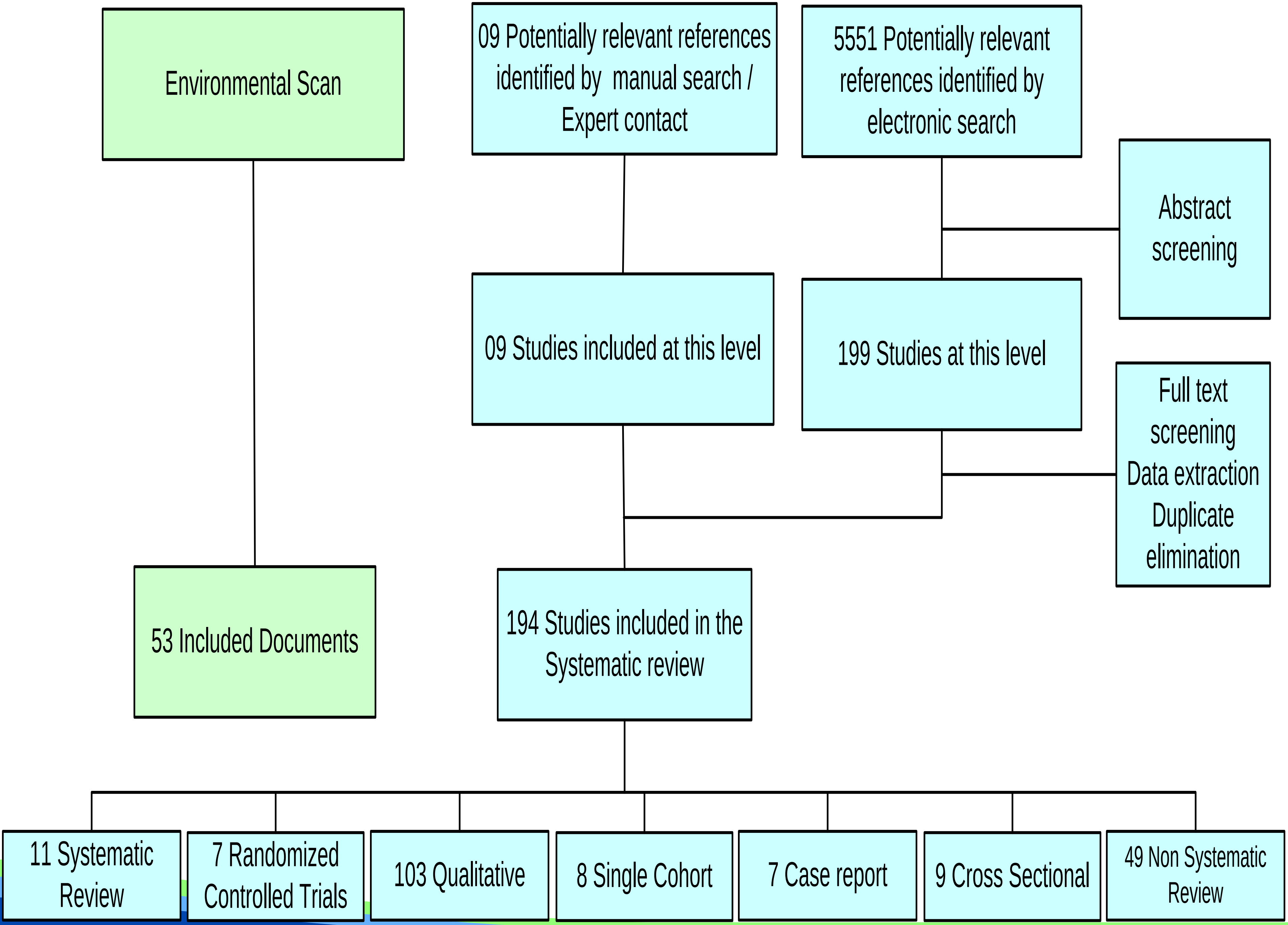
Study Design

- A systematic review:
  - Published biomedical literature
- Environmental scan:
  - Unpublished literature
  - Relevant actors, stakeholders, key events, groups, documentation
  - Non healthcare settings

Phases in meta-narrative review

- ***Planning phase***
- ***Search phase***
- ***Mapping phase***
- ***Appraisal phase***
- ***Synthesis phase***
- ***Recommendations phase***

Adapted from Greenlagh et al. Social Science & Medicine 61 (2005) 417–430



Environmental Scan

09 Potentially relevant references identified by manual search / Expert contact

5551 Potentially relevant references identified by electronic search

Abstract screening

09 Studies included at this level

199 Studies at this level

Full text screening
Data extraction
Duplicate elimination

53 Included Documents

194 Studies included in the Systematic review

11 Systematic Review

7 Randomized Controlled Trials

103 Qualitative

8 Single Cohort

7 Case report

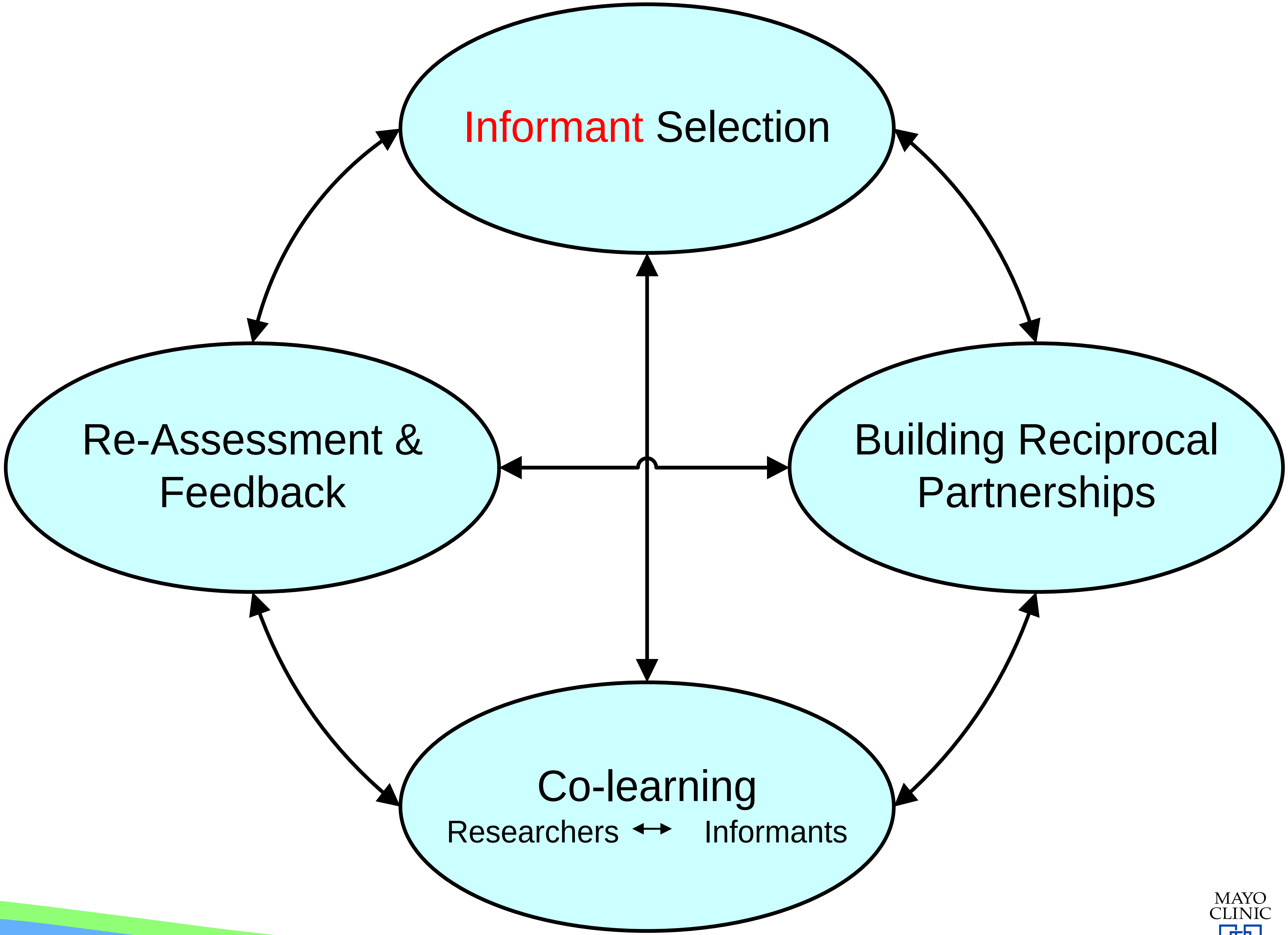
9 Cross Sectional

49 Non Systematic Review

Frameworks of engagement

Frameworks of engagement

- We found 34 studies that described a framework/ scheme/model that included steps for the process of patient engagement
- Models converged into 3 frameworks:
  - 4 common iterative steps for engagement
  - Engagement as a function of research stage
  - Potential spectrum of patient engagement in research



Informant Selection

Re-Assessment & Feedback

Building Reciprocal Partnerships

Co-learning

Researchers ↔ Informants

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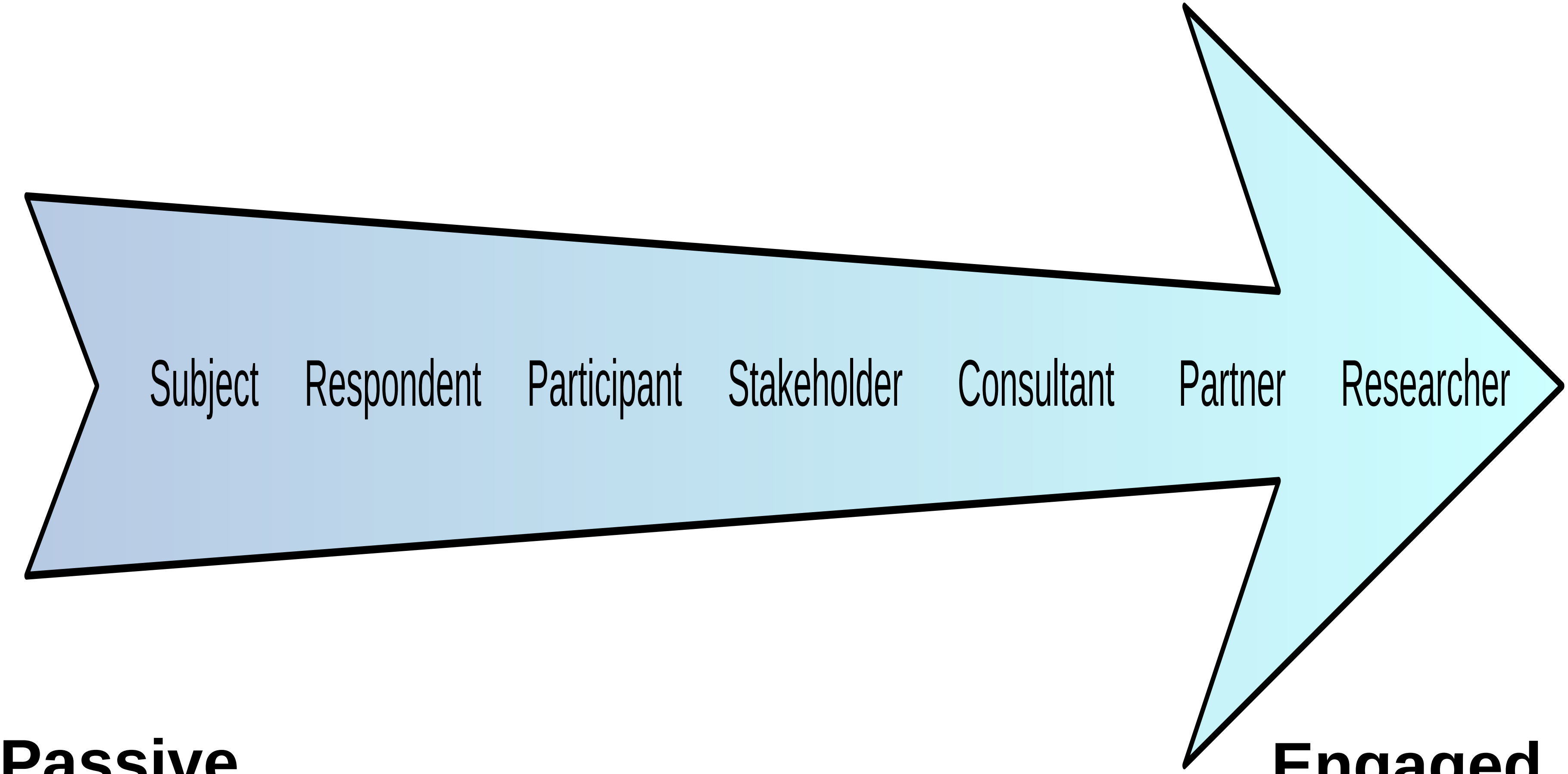
Stage of Informant Engagement

- Preparatory Stage
  - Agenda Setting & Funding
- Execution Stage
  - Study Design & Procedures
  - Study Recruitment
  - Data Collection
  - Data Analysis
- Translational Stage
  - Dissemination
  - Implementation
  - Evaluation



Subject Respondent Participant Stakeholder Consultant Partner Researcher

Passive

Engaged

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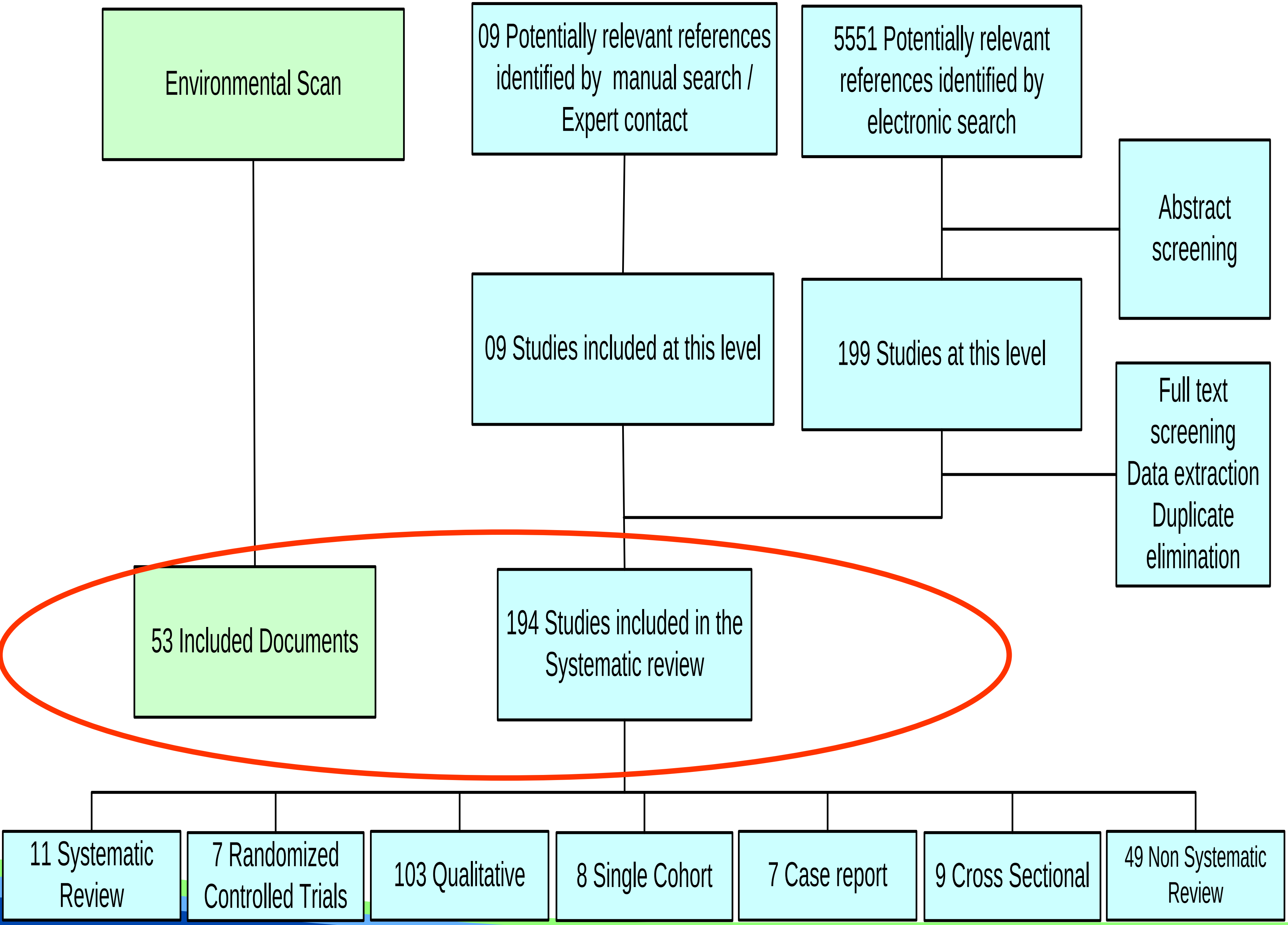
Results



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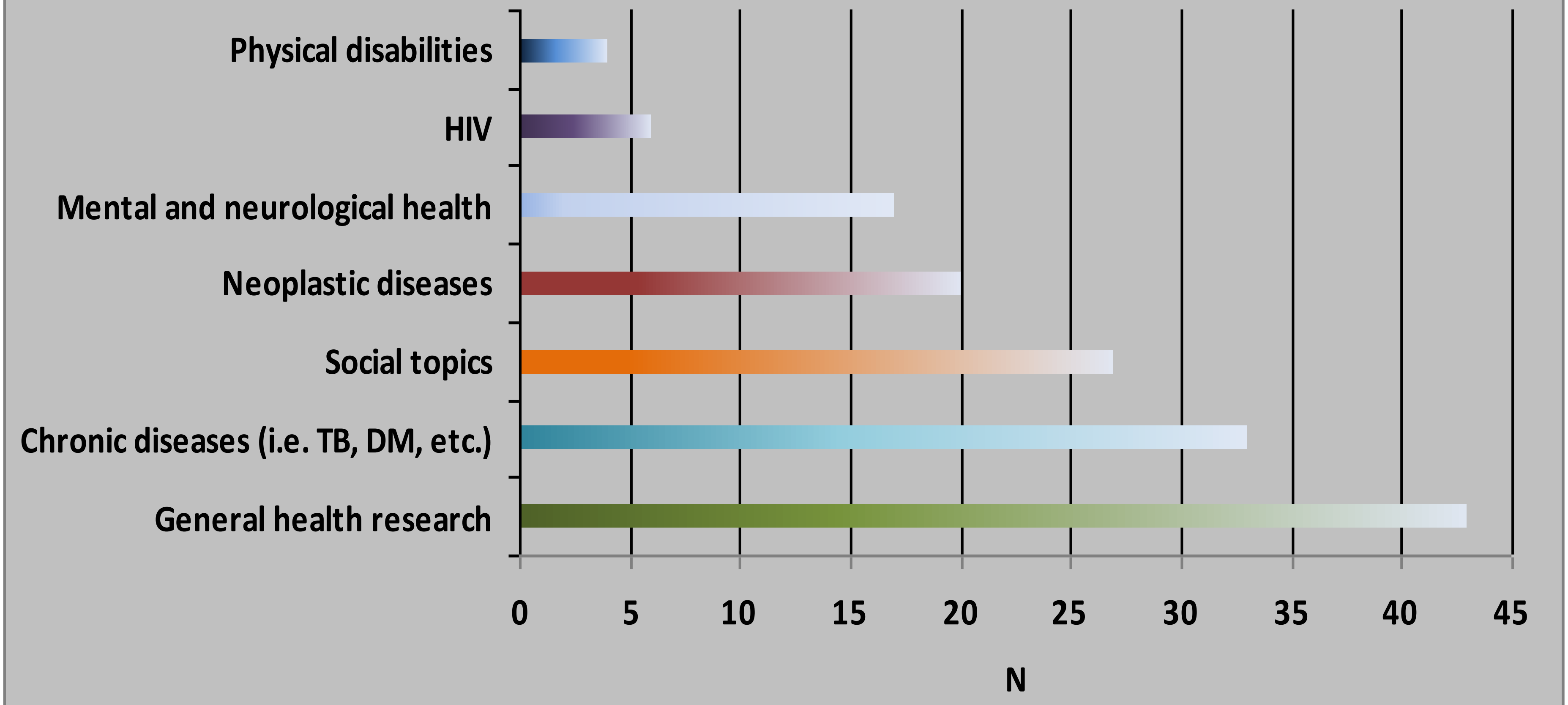
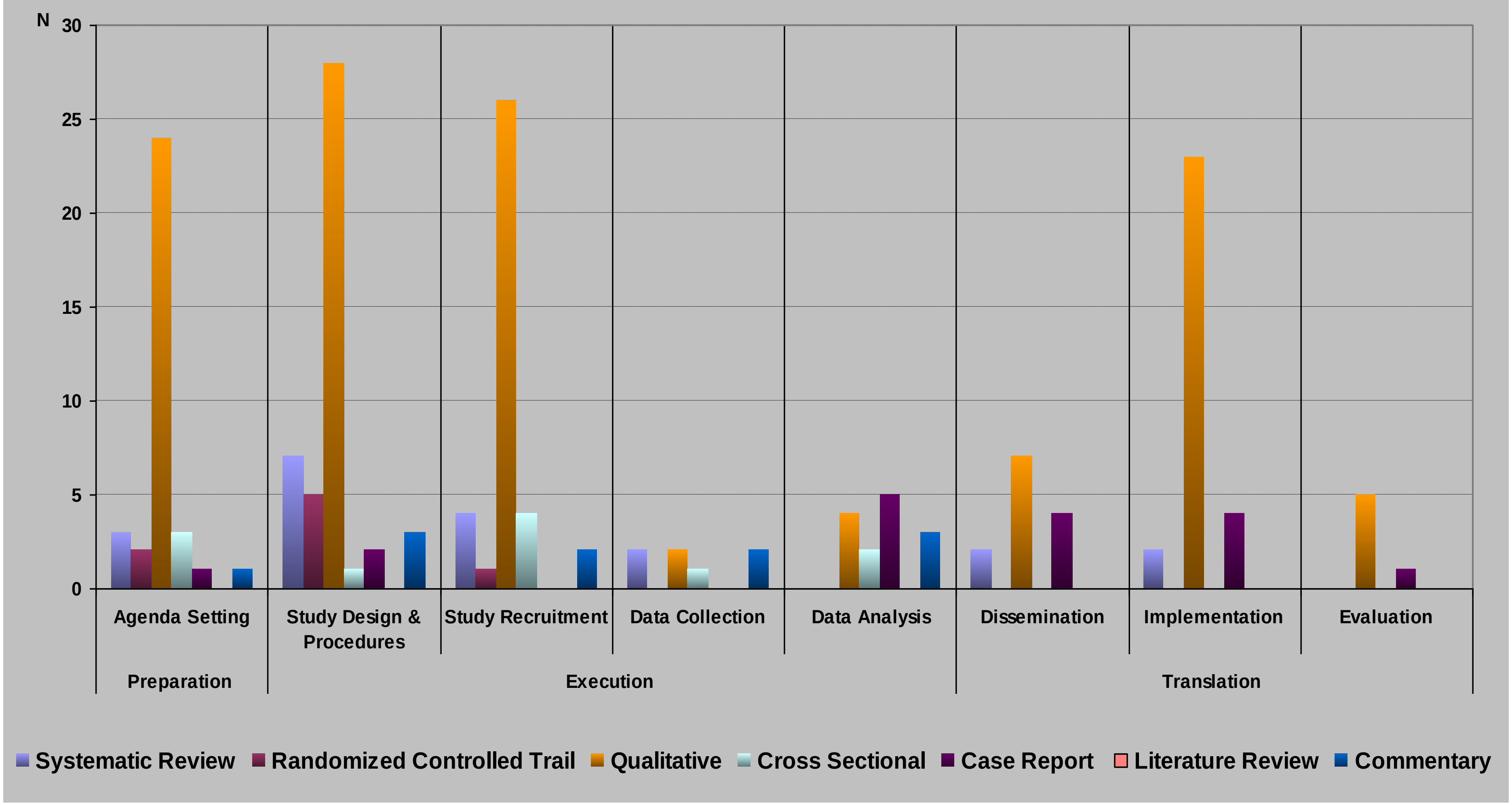
Study settings



Included Study Design



Example case studies

- 3 Studies from the systematic review
- 2 hits from the environmental scan

Example 1

- Semi-structured interviews conducted with 20 parents of children with cerebral palsy to evaluate 4 different trial designs, choice of outcome and reimbursement of participants
- Parents made choices
- Study reports higher enrollment and retention rates and several barriers

Edward et al. Health Expect. Dec 2011;14(4):429-438.

Example 2

- Mailed a questionnaire to a stratified random sample of 4,796 patients with diabetes
- Examine patients' preferences regarding the design of diabetes trials.
  - patient-important outcomes (vs. surrogate outcomes)
  - practical/pragmatic answers (vs. mechanistic/explanatory answers)?

Murad et al. / Journal of Clinical Epidemiology 64 (2011) 743-748

Example 3

- To develop a conceptual framework of PROs for metastatic Prostate Cancer
- Interview with 15 metastatic Prostate Cancer patients and a survey of 10 practitioners
- Patients endorsed (and practitioners confirmed) the relevance and importance of several symptoms, concerns and general domains of quality of life

Eton et al. Value in Health. 13(5): 2010. 613–623

Example 4

- Patient-initiated study
- Patients with amyotrophic lateral sclerosis experimented with lithium carbonate treatment (a therapy that has not received regulatory approval for their condition)
- Patients analyzed and reported their results on the website PatientsLikeMe.com

Example 5: INVOLVE/UK

- A national advisory group (30 members) created and funded by the National Institute of Health Research to supports greater public involvement in public health and social care research
- Identifies issues that need to be addressed by INVOLVE and disseminates new ideas on policy and practice
- Database of research projects that have/plan to actively involve members of the public as partners in the research process
- Opportunities for recruitment, training of patients

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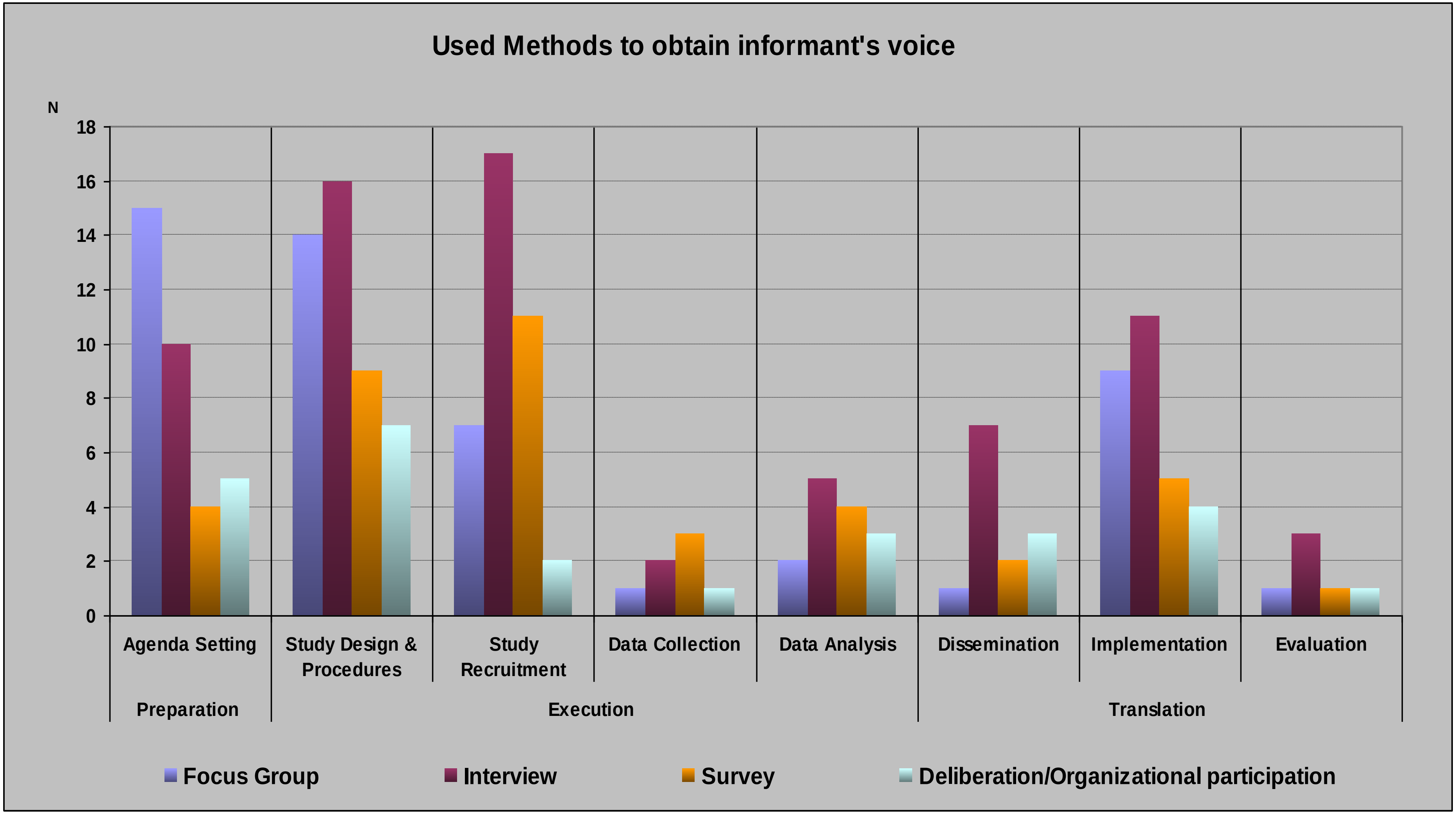
Methods of engagement in the literature

Selection

- Studies poorly described various methods
- Mostly self-selection out of a convenience sample
- Environmental scan:
  - Disease specific social networks
  - Networks designated for patient engagement (Europe/Canada)
- We did not find comparative studies to determine the relative efficacy of a particular method of identifying patient representatives



Used Methods to obtain informant's voice

Recommendations from the literature

Recommendation 1

Engaging patients and surrogates in all research phases (preparatory, execution and translation) is suggested and is feasible in most cases.
Potential risks for engaged patients seem to be minimal and outweighed by benefits:

- Patients' engagement as the ultimate user of research evidence is ethically and morally compelling
- Possible improvement in study design (outcome and intervention selection)
- Possible improvement in study execution (subject recruitment and retention)
- Possibly higher quality evidence (lower risk of bias)
- More applicable research

Recommendation 2

We recommend a framework for engaging informants that includes:

a. Proper and wide representation of the study population
b. Building a reciprocal partnership between researchers and informants that includes mutual respect and explicit expectations
c. A co-learning process (where researchers learn from the informants and vice versa) to be done throughout the whole engagement process and maintained during the study
d. The involvement process should be continually evaluated using predefined tools and possibly by external evaluators

Recommendation 3a

- We suggest selecting representatives that are as similar as possible to the community or population in which the study results are intended to be applied. This includes relevant ethnic minorities, elderly, young, disabled, incarcerated and any other special or vulnerable populations impacted by the research. Empiric evidence exists to suggest that the engagement of all these categories of patients or their surrogates is feasible in most cases.

Recommendation 3b

- We suggest that patient and surrogate engagement be initiated as early as possible in the research project and as frequent as feasible.

Recommendation 3c

- We suggest that the choice of methods for selecting patients or engaging them in research be made based on the research questions being asked and the overall aims of the research. We were unable to recommend a preferred strategy due to the lack of comparative data.

Patient Advisory Feedback

- No clear preferred term patient/informant
- Frameworks found very helpful
- Ranked first recommendation as most important
- Ranked the 4 steps of the framework as equally important
- Surprised of the possible extent of engagement
- Wordsmithing suggestions

Limitations

- Lack of comparison (recommendation for research)
- Poor indexing and reporting standards (recommendation for research/reporting)
- Publication bias/lack of denominator
- Multiple barriers identified
- Concern about tokenistic engagement

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Summary

- Patient engagement is suggested/potential benefits
- Framework presented, requires validation
- No comparative data/concern about bias
- Weak recommendations/?? standards