

POSTER PRESENTATION

Open Access

# How do clinical researchers' and patients' preferences influence study hypotheses and reported outcome results for clinical randomised controlled trials? A critical appraisal

Paola Rosati<sup>1,2\*</sup>, Roberto D'Amico<sup>3</sup>, Gabriella Ricciotti<sup>1</sup>, Giuseppina Testa<sup>1</sup>, Rita Inglese<sup>1</sup>, Ferruccio Giustini<sup>1</sup>, Ersilia Fiscarelli<sup>1</sup>, Marco Zazza<sup>1</sup>, Cecilia Carlino<sup>4</sup>, Valerio Balassone<sup>5</sup>, Roberto Fiorito<sup>5</sup>, Franz Porzolt<sup>6</sup>

From The 4th Meeting of the Core Outcome Measures in Effectiveness Trials (COMET) Initiative Rome, Italy. 19-20 November 2014

## Background

Studies designed to promote unbiased research increasingly show that human preferences exert a major influence on randomised controlled trials (RCTs) [1-3]. More information is needed on how preferences influence clinical trial design and conduction [4-6]. To fill the information gap between what researchers seek and report and what patients want [7,8], in this study we investigated how researchers' and patients' preferences influence study hypotheses and outcome results of published clinical RCTs. Because conventional critical appraisal seemed inappropriate for addressing our research question, in this pilot study we developed a novel assessment method and applied it in an RCT sample.

## Methods

We collected 20 unselected and consecutive RCTs published in a high impact paediatric journal from July to November 2013. Two experienced reviewers identified the following five domains and a grading method to score discrepancy between what author's state in clinical trial registries (CTRs) and report in published RCTs: reported funding (1 point), study hypotheses, information on patients enrolled and study conduction (3 points); primary and secondary outcomes, early study completion, and upgrading or downgrading outcome results (5 points).

Higher scores implied marked discrepancy. Two reviewers then independently applied the method on the RCT sample by mapping and coding information for the domains identified and reported discrepancies by comparing CTRs and RCTs (Table S1, Additional file 1).

## Results

Of the 20 RCTs collected and CTRs compared, 14 studies had high total preference discrepancy scores (7 scored 10-12, and 7 scored 16 or more) and 4 had discrepancy in declaring funding. In 12 studies researchers completed the study early and in 8 studies they downgraded or upgraded outcomes. Only 5 CTRs were updated but they neglected to include published RCT results. Only in 5 CTRs, dataset supervisors indicated the RCT URL. None of the 20 RCTs allowed us to assess patients' preferences (no information reported for non-response and refusal). No difference was found in discrepancy scores among the five CTR databases.

## Conclusions

The high discrepancy scores obtained by comparing what researchers stated in CTRs and published in RCTs suggest possible misconduct. Patients' preferences during RCT enrolment and conduction remain undetectable owing to the lack of targeted protocols to elicit this issue. These results, if confirmed in further studies, should prompt international regulation developers [2] to encourage researchers to explore patients' preferences as a strategy to enhance informed decision-making and to improve reporting in RCTs and CTRs.

\* Correspondence: paola.rosati@opbg.net

<sup>1</sup>G.A.L.I.L.E.O. Gruppo Apprezzamento Letteratura e Implementazione Livelli di Evidenza in Ospedale, Bambino Gesù Children's Hospital IRCCS, Rome, 00165, Italy

Full list of author information is available at the end of the article

## Additional material

Additional file 1:

### Authors' details

<sup>1</sup>G.A.L.I.L.E.O. Gruppo Apprezzamento Letteratura e Implementazione Livelli di Evidenza in Ospedale, Bambino Gesù Children's Hospital IRCCS, Rome, 00165, Italy. <sup>2</sup>Unit of Clinical Epidemiology, Bambino Gesù Children's Hospital IRCCS, Rome, 00165, Italy. <sup>3</sup>Italian Cochrane Center, Department of Diagnostic, Clinical and Public Health Medicine, University of Modena and Reggio Emilia, Modena, 41124, Italy. <sup>4</sup>Faculty of Medicine and Surgery, La Sapienza University, Rome, 00185, Italy. <sup>5</sup>General Surgery and Transplantation Department, University Tor Vergata, Rome, 00133, Italy. <sup>6</sup>Health Care Research, General and Visceral Surgery, University Hospital Ulm, Germany President, Institute of Clinical Economics (ICE) e.V., Ulm, 89070, Germany.

Published: 29 May 2015

### References

1. King M, Nazareth I, Lampe F, Bower P, Chandler M, Morou M, *et al*: **Impact of participant and physician intervention preferences on randomized trials: a systematic review.** *JAMA* 2005, **293**:1089-99.
2. **WHO Statement on public disclosure of clinical trial results.** *Call for public consultation* 2014 [<http://www.who.int/ictrp/results/en/>].
3. Walker E, Nowacki AS: **Understanding equivalence and noninferiority testing.** *J Gen Intern Med* 2011, **26**:192-196.
4. Stengel D, Sehouli J, Porzolt F: **Are the results of randomized trials influenced by preference effects? Part I. Findings from a systematic review.** In *Optimizing Health-Improving the Value of Healthcare Delivery*. New York: Springer; Porzolt F, Kaplan RM 2006:265-291.
5. Porzolt F, Stengel D: **Are the results of randomized trials influenced by preference effects? Part II. Why current studies often fail to answer this question.** In *Optimizing Health-Improving the Value of Healthcare Delivery*. New York: Springer; Porzolt F, Kaplan RM 2006:292-297.
6. Ioannidis JPA, Greenland S, Hlatky MA, Khoury MJ, Macleod MR, Moher D, *et al*: **Increasing value and reducing waste in research design, conduct and analysis.** *Lancet* 2014, **383**:166-75.
7. Wade J, Donovan JL, Lane JA, Neal DE, Hamdy FC: **It's not just what you say, it's also how you say it: opening the 'black box' of informed consent appointments in randomised controlled trials.** *Soc Sci Med* 2009, **68**:2018-2028.
8. Mills N, Donovan JL, Wade J, Hamdy FC, Neal DE, Lane JA: **Exploring treatment preferences facilitated recruitment to randomized controlled trials.** *J Clin Epidemiol* 2011, **64**:1127-1136.

doi:10.1186/1745-6215-16-S1-P34

**Cite this article as:** Rosati *et al*: How do clinical researchers' and patients' preferences influence study hypotheses and reported outcome results for clinical randomised controlled trials? A critical appraisal. *Trials* 2015 **16**(Suppl 1):P34.

Submit your next manuscript to BioMed Central  
and take full advantage of:

- Convenient online submission
- Thorough peer review
- No space constraints or color figure charges
- Immediate publication on acceptance
- Inclusion in PubMed, CAS, Scopus and Google Scholar
- Research which is freely available for redistribution

Submit your manuscript at  
[www.biomedcentral.com/submit](http://www.biomedcentral.com/submit)

