Effect Size in the Design and Interpretation of Randomized Trials: A (So-Called) Statistical Reviewer's Perspective

Supplementary References

Overviews of approaches to setting a target effect size

- Cook JA, Hislop J, Adewuyi TE, Harrild K, Altman DG, Ramsay CR, Fraser C, Buckley B, Fayers P, Harvey I, Briggs AH, Norrie JD, Fergusson D, Ford I, Vale LD. Assessing methods to specify the target difference for a randomised controlled trial: DELTA (Difference ELicitation in TriAls) review. Health Technol Assess 2014; 18:v–vi, 1–175.
- Hislop J, Adewuyi TE, Vale LD, Harrild K, Fraser C, Gurung T, Altman DG, Briggs AH, Fayers P, Ramsay CR, Norrie JD, Harvey IM, Buckley B, Cook JA, DELTA group. Methods for specifying the target difference in a randomised controlled trial: the Difference ELicitation in TriAls (DELTA) systematic review. PLoS Med 2014; 11:e1001645.

Studies of actual reporting practices

- Lee PH, Tse ACY. The quality of the reported sample size calculations in randomized controlled trials indexed in PubMed. Eur J Intern Med 2017; 40:16–21.
- Charles P, Giraudeau B, Dechartres A, Baron G, Ravaud P. Reporting of sample size calculation in randomised controlled trials: review. BMJ 2009; 338:b1732.
- Chan KB, Man-Son-Hing M, Molnar FJ, Laupacis A. How well is the clinical importance of study results reported? An assessment of randomized controlled trials. CMAJ 2001; 165:1197–1202.
- Moher D, Dulberg CS, Wells GA. Statistical power, sample size, and their reporting in randomized controlled trials. JAMA 1994; 272:122–124.

Minimum clinically important difference

- McGlothlin AE, Lewis RJ. Minimal clinically important difference: defining what really matters to patients. JAMA 2014; 312:1342–1343.
- Gatchel RJ, Lurie JD, Mayer TG. Minimal clinically important difference. Spine 2010; 35:1739–1743.

- Dworkin RH, Turk DC, Wyrwich KW, Beaton D, Cleeland CS, Farrar JT, Haythornthwaite JA, Jensen MP, Kerns RD, Ader DN, Brandenburg N, Burke LB, Cella D, Chandler J, Cowan P, Dimitrova R, Dionne R, Hertz S, Jadad AR, Katz NP, Kehlet H, Kramer LD, Manning DC, McCormick C, McDermott MP, McQuay HJ, Patel S, Porter L, Quessy S, Rappaport BA, Rauschkolb C, Revicki DA, Rothman M, Schmader KE, Stacey BR, Stauffer JW, von Stein T, White RE, Witter J, Zavisic S. Interpreting the clinical importance of treatment outcomes in chronic pain clinical trials: IMMPACT recommendations. J Pain 2008; 9:105–121.
- Copay AG, Subach BR, Glassman SD, Polly DW Jr, Schuler TC. Understanding the minimum clinically important difference: a review of concepts and methods. Spine J 2007; 7:541–546.

Proper and improper roles of pilot studies

- Kistin C, Silverstein M. Pilot studies: a critical but potentially misused component of interventional research. JAMA 2015; 314:1561–1562.
- Whitehead AL, Sully BGO, Campbell MJ. Pilot and feasibility studies: is there a difference from each other and from a randomised controlled trial? Contemp Clin Trials 2014; 38:130–133.
- Leon AC, Davis LL, Kraemer HC. The role and interpretation of pilot studies in clinical research. J Psychiatr Res 2011; 45:626–629.
- Kraemer HC, Mintz J, Noda A, Tinklenberg J, Yesavage JA. Caution regarding the use of pilot studies to guide power calculations for study proposals. Arch Gen Psychiatry 2006; 63:484–489.

(Mis-)interpretation of "negative" trials

- Gewandter JS, McDermott MP, Kitt RA, Chaudari J, Koch JG, Evans SR, Gross RA, Markman JD, Turk DC, Dworkin RH. Interpretation of CIs in clinical trials with non-significant results: systematic review and recommendations. BMJ Open 2017; 7:e017288.
- Leontiadis GI. How to interpret a negative study. Am J Gastroenterol 2016; 111:1506–1507.
- Altman DG, Bland JM. Absence of evidence is not evidence of absence. BMJ 1995; 311:485.
- Moher D, Dulberg CS, Wells GA. Statistical power, sample size, and their reporting in randomized controlled trials. JAMA 1994; 272:122–124.

• Freiman JA, Chalmers TC, Smith H Jr, Kuebler RR. The importance of beta, the type II error and sample size in the design and interpretation of the randomized control trial. Survey of 71 'negative' trials. N Engl J Med 1978; 299:690–694.

Pitfalls of post hoc power calculations

- Schulz KF, Grimes DA. Sample size calculations in randomised trials: mandatory and mystical. Lancet 2005; 365:1348–1353.
- Hoenig JM, Heisey DM. The abuse of power: the pervasive fallacy of power calculations for data analysis. Amer Statistician 2001; 55:19–24.
- Goodman SN, Berlin JA. The use of predicted confidence intervals when planning experiments and the misuse of power when interpreting results. Ann Intern Med 1994; 121:200–206.
- Tukey JW. Tightening the clinical trial. Control Clin Trials 1993; 14:266–285.

CONSORT guidelines, explained and justified

• Moher D, Hopewell S, Schulz KF, Montori V, Gøtzsche PC, Devereaux PJ, Elbourne D, Egger M, Altman DG. Consolidated Standards of Reporting Trials Group. CONSORT 2010 explanation and elaboration: updated guidelines for reporting parallel group randomised trials. J Clin Epidemiol 2010; 63:e1–e37.