Towards improving quality of the evidence base for medical decision-making

Jansen, M.S.

Stellingen
behorende bij het proefschrift

Towards improving quality of the evidence base for medical decision-making

1. "New research should not be undertaken unless, at its initiation, the questions it proposes to address cannot be answered satisfactorily with existing evidence." Research protocols should contain a systematic assessment of existing evidence and ongoing studies to justify the need for new research. *(Adapted from Chalmers and Glasziou, Lancet, 2009)*
2. The choice and assessment of sample size parameters, particularly the target difference, require subject-matter expertise. The evaluation of sample size calculations by research ethics committees should not rest solely with statisticians, but also involve clinicians to judge the plausibility and relevance of chosen effect sizes. *(This thesis)*
3. About one-third of clinical trials are discontinued prematurely, often due to recruitment failure, which is particularly common in investigator-initiated trials. Research ethics committees should consider incorporating systematic feasibility assessments as a standard element of ethics review to help reduce the number of trials that stop early due to poor recruitment. *(This thesis)*
4. Many trials that do not recruit their target sample size fail to disseminate their results. Yet even such trials should be published, as their findings can contribute to systematic reviews or meta-analyses that ultimately inform clinical practice. "Unbiased trials with imprecise results trump no results at all." *(This thesis, adapted from Schulz and Grimes, Lancet, 2005)*
5. Research ethics committees are uniquely positioned to help ensure universal dissemination of trial results. *(This thesis)*
6. "A P value between 0.001 and 0.005 should not be taken as confirmation that a study was highly powered for the true effect it sought to estimate." As many trials are underpowered relative to the true effect they aim to estimate, it is unsurprising that significant, or even close to significant findings are often inflated and difficult to reproduce in subsequent studies. *(Adapted from van Zwet et al., NEJM Evidence, 2023)*
7. "Traditional randomised controlled trials can only answer a minuscule fraction of the near-infinite number of questions about subpopulations, interactions, treatment settings, and effects, that are relevant to patients and clinicians in every day care." High quality real-world evidence is thus an essential complement to traditional trials, as it can address important questions that would otherwise remain unanswered. *(Adapted from Eichler et al., Clinical Pharmacology & Therapeutics 2021)*
8. Regulatory and health technology assessment guidance should not only specify when real world evidence regarding treatment effectiveness is acceptable and desirable, but also when it is insufficient to inform decision-making if used as standalone evidence. In other words, it should delineate when evidence from traditional randomised trials remains a minimum requirement. *(This thesis)*
9. There are many parallels between scientific research and renovating a house: both ideally begin with a good plan and strong foundations. Without them, the final outcome is unlikely to stand the test of time.