

How Often Are Patient-Important Outcomes Represented in Neonatal Randomized Controlled Trials? An Analysis of Cochrane Neonatal Reviews

Nai Ming Lai^{a,b} Denise Yin Xian Leom^c Wen Li Chow^d Kee-Hsin Chen^{e–g}
Pu-Hong Lin^f Nathorn Chaiyakunapruk^h Colleen Ovelmanⁱ Roger Soll^{i,j}

^aSchool of Medicine, Faculty of Health and Medical Sciences, Taylor's University, Kuala Lumpur, Malaysia; ^bSchool of Pharmacy, Monash University, Kuala Lumpur, Malaysia; ^cDepartment of Emergency and Trauma, Labuan Hospital, Labuan, Malaysia; ^dDepartment of Paediatrics, Hospital Sultanah Nora Ismail, Batu Pahat, Batu Pahat, Malaysia; ^ePost-Baccalaureate Program in Nursing, College of Nursing and Cochrane Taiwan, Taipei Medical University, Taipei, Taiwan; ^fDepartment of Nursing, Wan Fang Hospital, Taipei Medical University, Taipei, Taiwan; ^gEvidence-Based Knowledge Translation Center, Wan Fang Hospital, Taipei Medical University, Taipei, Taiwan; ^hDepartment of Pharmacotherapy, College of Pharmacy, University of Utah, Salt Lake City, UT, USA; ⁱCochrane Neonatal, Burlington, VT, USA; ^jDivision of Pediatrics – Neonatology, The University of Vermont Medical Center, Burlington, VT, USA

Keywords

Neonatal outcome · Randomized controlled trial · Patient-important outcomes

Abstract

Background: Research findings based on patient-important outcomes (PIOs) provide more useful conclusions than those that are based on surrogate outcomes. It is unclear to what extent PIOs are represented in neonatal randomized controlled trials (RCTs). **Objectives:** We determined the proportion of PIOs in neonatal RCTs included in Cochrane Neonatal reviews. **Methods:** We extracted up to 5 outcomes from each RCT included in Cochrane Neonatal reviews published until January 2018, with independent determination of PIOs among authors followed by a discussion leading to a consensus. We defined PIOs as outcomes that matter to patient care, such as clinical events or physiological or laboratory parameters that are widely used to guide management. **Results:** Among 6,832 outcomes extracted from 1,874 RCTs included in 276 reviews, 5,349 (78.3%) were considered PIOs; 461 studies (24.5%) included 5 or more PIOs, 1,278 (68.2%)

included 1–4 PIOs, while 135 (7.2%) had no PIO included. PIOs were observed more often among dichotomous than among continuous outcomes (94.9 vs. 61.5%; RR: 1.54; 95% CI: 1.50–1.58), and more among subjective than among objective outcomes (95.9 vs. 76.8%; RR: 1.25; 95% CI: 1.22–1.28). Newer studies were more likely to have a greater number of PIOs (adjusted OR: 1.033 [95% CI: 1.025–1.041] with each publication year). **Conclusions:** The large and increasing representation of PIOs over the years suggests an improving awareness by neonatal trialists of the need to incorporate important outcomes in order to justify the utilization of resources. Further research should explore the reasons for non-inclusion or non-reporting of PIOs in a small proportion of RCTs.

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Background

A clinical trial that is useful for informing practice should have credible methodologies [1], good quality of reporting [2], and findings that are interpretable and ap-