Review of an innovative approach to practical public and clinical health trials

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Background

The ‘cohort multiple randomised controlled trial’ (cmRCT) is an innovative approach to the design and conduct of comparative effectiveness trials (Relton et al 2010). The design utilises a large observational cohort of patients with the condition of interest with regular measurement of outcomes for the whole cohort. This provides a facility for multiple randomised controlled trials (RCTs) embedded within the cohort, public health and/or clinical trials. Information and consent processes aim to replicate those in routine healthcare.

Methods

Information was identified through citations of the original article (Relton et al 2010) augmented by trial expertise networks. Studies were included if they had protocols which cited the cmRCT design and had relevant approvals for the establishment of the cohort. Data were extracted from study protocols, articles and presentations describing the rationale for using the cmRCT design.

Results

16 studies implementing the cmRCT design were identified in the UK, Canada and The Netherlands in both public health and clinical settings. Cohort sizes varied from 345 to 22,706. The condition of interest for each cohort either focussed on a single disease or injury (e.g. hip fracture, degenerative knee injury, scleroderma, breast cancer, colorectal cancer, bone metastases, homeopathy), or had a broader focus (e.g. populations at risk of falls, frailty, long term conditions, depression, mental health problems). Within these 16 cohorts 18 ongoing or completed trials were embedded.

Conclusions

This is the first review of the cmRCT design in practice and brings together the experiences of Canadian, Dutch and UK researchers implementing the design. Due to the challenges of identifying studies in their early stages, not all studies using or planning to use the cmRCT design have necessarily been identified. The description of the 16 studies identified will serve as a guide to researchers interested in using the cmRCT design. Future research needs to (i) assess if/when this design is preferable to the standard approach to RCT design, (ii) explore whether the design could be applied to existing cohorts and (iii) monitor the potential of this design to facilitate healthcare trials of all varieties.

Key messages

- The ‘cohort multiple randomised controlled trial’ (cmRCT) is an innovative approach to the design and conduct of comparative effectiveness trials in public health and/or clinical health
- This review of early implementation of the design demonstrates some acceptability of the design in a wide range of settings in the UK, Canada and the Netherlands