Social Science & Medicine 200 (2018) 114–128

Contents lists available at ScienceDirect

Social Science & Medicine

journal homepage: www.elsevier.com/locate/socscimed

Review article

A systematic review of the methodological and practical challenges of undertaking randomised-controlled trials with cognitive disability populations

Peter Mulhall[a,*], Laurence Taggart[a], Vivien Coates[a], Toni McAloon[a], Angela Hassiotis[b]

[a] School of Nursing, Ulster University, Shore Rd, Newtownabbey, BT37 0QB, Northern Ireland, United Kingdom
[b] University College London, Division of Psychiatry, 6th Floor, Maple House, 149 Tottenham Court Road, London, W1T 7NF, United Kingdom

ARTICLE INFO

Keywords:
Barriers
Randomised controlled trial
Evaluation
Cognitive disability
Intellectual disability
Evidence base
Experience base

ABSTRACT

Approximately 10% of the world's population have a cognitive disability. Cognitive disabilities can have a profound impact on a person's social, cognitive or mental functioning, requiring high levels of costly health and social support. Therefore, it is imperative that interventions and services received are based upon a sound evidence-base. For many interventions for this population, this evidence-base does not yet exist and there is a need for more Randomised Controlled Trials (RCTs). The process of conducting RCTs with disabled populations is fraught with methodological challenges. We need a better understanding of these methodological barriers if the evidence-bases are to be developed. The purpose of this study was to explore the methodological and practical barriers to conducting trials with adults with cognitive disabilities. As a case example, the literature regarding RCTs for people with intellectual disabilities (ID) was used to highlight these pertinent issues. A systematic literature review was conducted of RCTs with adults with ID, published from 2000 to 2017. A total of 53 papers met the inclusion criteria and were reviewed. Some of the barriers reported were specific to the RCT methodology and others specific to people with disabilities. Notable barriers included; difficulties recruiting; obtaining consent; resistance to the use of control groups; engaging with carers, staff and stakeholders; the need to adapt interventions and resources to be disability-accessible; and staff turnover. Conducting RCTs with people with cognitive disabilities can be challenging, however with reasonable adjustments, many of these barriers can be overcome. Researchers are not maximising the sharing of their experience-base. As a result, the development of evidence-bases remains slow and the health inequities of people with disabilities will continue to grow. The importance of the MRC guidelines on process evaluations, together with implications for the dissemination of 'evidence-base' and 'experience-base' are discussed.

1. Introduction

Globally about 10% of the world's population, approximately 650 million people, live with a disability (UN Fact sheet on Persons with Disabilities), many of whom have a cognitive disability. Cognitive disability can have many causal factors (e.g. stroke, dementia, acquired-brain injury, autistic spectrum disorder, intellectual disability) and can arise at different stages of life. Although the list of disorders that feature cognitive impairment may seem diverse, there is significant overlap amongst disabilities in how impairment may impact on a person's quality of life and their ability to function independently. For example, disruption of family life, reduced social activities and social isolation are commonly experienced by people with various developmental, and acquired, cognitive disabilities including dementia, stroke and autism (Giebel et al., 2014; Northcott et al., 2016; Spain and Blainey, 2015).

A clear exemplar of a common cognitive disability is the case of people with intellectual disabilities (ID). ID is a class of disorders with a range of genetic, biological and psycho-social aetiologies. The two most commonly used systems for diagnosing an ID are the American Psychiatric Association's Diagnostic Statistical Manual (DSM) and the World Health Organisation's International Classification of Diseases (ICD). Traditionally ID was often diagnosed when a person's IQ fell below two standard deviation below the mean (i.e. < 70). More recently DSM-V determines ID as being based more upon functioning level than IQ level. People with ID will have difficulties in intellectual functioning (such as problem-solving, planning, abstract thinking, reasoning, an IQ < 70 ± 5). They will also have difficulties in

* Corresponding author. School of Nursing, Ulster University, Shore Rd, Newtownabbey, BT37 0QB, Northern Ireland, United Kingdom.
E-mail address: mulhall-p@ulster.ac.uk (P. Mulhall).

https://doi.org/10.1016/j.socscimed.2018.01.032
Received 8 September 2017; Received in revised form 22 January 2018; Accepted 23 January 2018

adaptive functioning.

(i.e. self-care, domestic skills, social skills, self-direction, community, academic skills, work, leisure, health and safety); all occurring during development. The World Health Organisation's ICD sees intellectual disability as "a group of developmental conditions characterized by significant impairment of cognitive functions, which are associated with limitations of learning, adaptive behaviour and skills", with IQ being only one clinical marker for helping to determine 'severity' of the disability – the ICD further classifies ID into mild, moderate, severe and profound, largely on the basis of IQ and functioning (Salvador-Carulla et al., 2011). Approximately 1%–2% of the world's population have an ID, which amounts to about 15 million people in Europe alone (http://www.euractiv.com/sections/health-consumers/people-intellectual-disabilities-eu-deserve-proper-healthcare-310015); and it is predicted that this population will grow. Likewise, population growth with the other cognitive disability populations is also predicted. For example, the global prevalence of dementia is expected to double every twenty years (Mavrodaris et al., 2013).

Despite people with ID living longer (Braddock et al., 2013), recent research in the UK, Ireland, USA and Australia highlights that they are dying approximately 20 years earlier than non-ID peers from respiratory disease, coronary heart disease and specific cancers (Heslop et al., 2013; McCarron et al., 2015; Trollor et al., 2017; US Surgeons General Report, 2002). Furthermore, people with ID have higher prevalence rates of a range of secondary chronic health conditions (i.e. sensory problems, epilepsy, Type 2 diabetes, osteoporosis, mental health, dementia) compared to the non-ID population (Taggart and Cousins, 2014). Alongside this, there is growing international evidence to show that many of these health inequalities can be avoided with appropriate health surveillance, health screening, early interventions and effective clinical interventions (Emerson and Hatton, 2013; Heslop et al., 2013; Taggart and Cousins, 2014). An important distinction can be made then between the unavoidable health inequality faced by people with ID due to the genetic nature of their disability, and the avoidable health inequities that they face due to inappropriate, inadequate or absent provision of services and care.

The global costs of providing primary/secondary healthcare and social care for those with ID, and other cognitive disabilities, is estimated to cost countries a substantial proportion of their overall fiscal budgets and is becoming unsustainable (Pavolini and Ranci, 2008; Wimo and Prince, 2010). For example, in the UK, older adults with ID account for 0.15–0.25% of the population, however they receive up to 5% of the total personal care budget (Strydom et al., 2010). The National Audit Office Report (2017) for the Department of Health reports that in England £8 billion are spent providing services to people with ID. In the Netherlands ID expenditure accounted for 9% of the total healthcare expenditure (Polder et al., 2002). Given the austerity measures many countries face today, it is imperative that pharmacological and psycho-social interventions are both clinically effective and cost-effective (Robertson et al., 2015) and are supported by a strong evidence-base.

In the non-disabled population, the evidence-base for many pharmacological and psycho-social interventions is informed by large scale randomised control trials (RCTs) and systematic reviews/meta analyses. RCTs are widely considered the 'gold standard' for testing the effectiveness of treatment interventions. This is in part because RCTs offer levels of rigour that many other methodologies lack. The three central principles of the RCT methodology are Randomisation, Control and Trial or testing of an intervention (see Fig. 1):

- **Randomisation:** a representative sample of the population is randomly assigned to either an intervention or a control group;
- **Control**: measures are taken to reduce the influence of extraneous variables to isolate and examine the effect of the intervention under investigation;
- **Trial**: a treatment or intervention is tested within a specified framework to assess its effectiveness and/or efficiency. This requires a well-defined, and adhered to, protocol; the use of appropriate outcome measures; and the use of appropriate statistical methods.

At first glance, the RCT methodology may appear deceptively simple. However, each of the three central principles of an RCT has its own unique methodological and practical challenges and levels of complexity. This complexity is magnified when incorporating participants with cognitive and communication difficulties, such as those with dementia, stroke, autism or ID. It could be argued that the RCT methodology is well suited to trials that test the *efficacy* of pharmacological interventions, e.g. does molecule A have a better impact than molecule B ***under optimal conditions***. However, many researchers are less convinced that the methodology should be used to test the *effectiveness* of behavioural or psychological interventions, which are often effected by the myriad potential interactions between people ***under real-world conditions***. As Hallfors and Cho (2007) state:

> "We argue that research has followed too closely after the pharmaceutical and medical product research model, with reliance on small efficacy trials under optimal conditions. While efficacy trials may be appropriate for medical product testing, they are not the best method for behavioural intervention research. Real world feasibility testing is essential, and external validity must become as important as internal validity for evidence of effectiveness." (p244-245)

Despite Hallfors & Cho's warning, there is still a heavy reliance in Evidence-Based Practice upon RCTs. A common occurrence in both systematic reviews and meta-analyses within the ID field is the statement that there is a dearth of evidence in the form of high quality RCTs (Koslowski et al., 2016; Sohanpal et al., 2007; Vereenooghe and Langdon, 2013). As such, the development of evidence-bases within the ID field lags considerably behind the non-disabled fields (Hastings, 2013). RCTs in the ID field remain uncommon and many have been fraught with methodological and practical challenges, and shortcomings. Not only are disability-specific trials uncommon, but it has also been shown that many people with cognitive disabilities, and particularly ID, are routinely excluded from 'mainstream' clinical trials (Brooker et al., 2015). Feldman et al. (2014) in a review of 300 randomly chosen RCTs found that people with ID were included in only 2% of these studies: with over 90% automatically excluding people with ID. Common RCT exclusion criterion included: language difficulties and/or cognitive impairment or inability to follow the intervention protocol. This further highlights the negative attitudes and on-going discriminatory practices that people with ID face. If the evidence-base for pharmacological and psycho-social interventions for people with ID is to be developed, then clearly a way must be found to either facilitate the inclusion of people with cognitive disabilities in mainstream RCTs, or more disability-specific trials must be commissioned and funded.

Whilst the generation of evidence from RCTs and systematic reviews is important, so too is the appropriate sharing and reporting of this evidence. The CONSORT statement (Schulz et al., 2010) proposes best practice and standardises the reporting of RCTs to ensure that important information is presented in such a way that readers can use the information to inform their decision-making and could, if required, replicate the study. The CONSORT statement is a 25-item checklist focusing on how the trial was designed, conducted, analysed, interpreted and has been adopted as a framework of best practice reporting in many peer reviewed journals across health and social care research fields. There are several variations of the CONSORT to accommodate different trial designs, interventions and data types (see http://www.consort-statement.org/extensions). Although frameworks such as CONSORT provide guidance on how to report on the **'procedure'** of a trial, they do not require reporting on the **'process'** of the trial. For example, authors are prompted to report the methodological steps undertaken in conducting the trial, and provide a measure of outcome but