Single case experimental design: a rigorous method for addressing inequity and enhancing precision within Para sport and exercise medicine research

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Approximately 4400 athletes from 184 nations competed in 22 sports at the 2024 Paris Paralympic Games. However, it is recognised that athletes with more severe disabilities and high support needs are under-represented in sport, and strategies to increase representation are required. Focusing on individuals with cerebral palsy (CP), we present evidence that people with high support needs are also under-represented in Para sport and exercise medicine (P-SEM) research. We outline why single case experimental designs (SCEDs) are a rigorous and effective means of addressing under-representation in P-SEM research.

CEREBRAL PALSY

CP is an eligible underlying health condition for 17 of the 22 Paralympic sports. It results from a non-progressive brain lesion and is defined as a heterogeneous group of permanent disorders affecting movement and posture.[1] CP heterogeneity is multidimensional and can be classified based on:

- Neurological subtype: Spastic CP (quadriplegia/diplegia/hemiplegia); dyskinetic; ataxic; and mixed.[2] Subtypes vary in severity and anatomical distribution.
- Functional effects: The Gross Motor Function Classification System (GMFCS) is the most common and has five levels: GMFCS level I (least severe) and II are able to walk independently; GMFCS IV/V use wheeled mobility and typically have high support needs (CP-HSN).

Additionally 95% of people with CP have >1 comorbidity[2] but CP-HSN have a greater prevalence of serious comorbidity including cortical blindness, deafness, gavage feeding and seizure disorders.[3]

EXERCISE TRAINING RESEARCH IN PEOPLE WITH CP

At least three major reviews have analysed randomised controlled trials (RCTs) that evaluate exercise training in people with CP and all report that CP-HSN participants are under-represented.[4–6] CP-HSN constitutes approximately 30% of the CP population[7] but <3% of study participants in sport and leisure intervention research.[4]

Several factors may contribute to this under-representation: increased incidence of serious comorbidities increases the risk of adverse events, requiring more stringent risk management measures, and more qualified staff; low physical function leads to relatively greater dependence on assistive technology, personal support workers and supported transport, thereby increasing the time and financial costs of research[8]; and there is a lack of valid and reliable physical activity metrics in people with severe physical impairments.

In addition to these practical considerations, it is plausible that under-representation of CP-HSN may indicate that researchers implicitly acknowledge that group-based designs such as RCTs are not appropriate n for this population. Specifically, within-population variability is particularly high in CP-HSN due to the high incidence and severity of comorbidities such as intellectual disability, seizure disorders, gavage feeding, fatigue and pain. These will often require adaptation to training protocols and introduce variability that increases statistical error (ie, reduces signal:noise ratio), thereby compromising internal validity to an unacceptable extent.

Importantly, randomisation of participants does not address the threat to internal validity posed by such heterogeneity. The aim of randomisation is to balance out differences between experimental and control groups by ensuring non-intervention-related factors are distributed between groups. This makes the groups otherwise comparable, except for exposure to the intervention, permitting the intervention effect to be detected. However, the variations in neurological subtype and comorbidities that characterise CP-HSN will influence individual exercise training protocols and training responses in ways that cannot be assumed to be equivalent (eg, the effect of intellectual disability vs gavage feeding). Furthermore, these prognostic variables are likely to interact in complex ways. For any given individual with CP-HSN, their unique mix of neurological subtype/s and comorbidity type and severity will affect exercise training responses in ways that are not sufficiently predictable to permit the confident recruitment of a homogeneous sample for randomisation. Therefore, it cannot be assumed that randomisation will be effective.

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GREATER PRECISION IS REQUIRED

The advent of precision healthcare has benefitted a range of patient groups which appear homogeneous with respect to observable signs and symptoms, but which can be divided into distinct subgroups based on differences at the genetic/molecular level (eg, people with various cancer types). This has permitted development of interventions that are more precise and effective. The use of RCTs to evaluate interventions for people with CP-HSN is the antithesis of a precision healthcare approach because it requires grouping people who are manifestly heterogeneous with respect to clinically important and observable signs and symptoms in order to achieve sufficiently large samples. Therefore, alternative research designs that facilitate precision are required.

SINGLE CASE EXPERIMENTAL DESIGNS (SCEDS)

SCEDs are study designs developed specifically to assess intervention effectiveness in populations that are highly complex and heterogeneous. In P-SEM research SCEDs involve repeated measures of the dependent variable/s (eg, strength, aerobic capacity, motor function) under different experimental conditions (eg, exercising or not exercising) within a single case (eg, a Para athlete or patient with high support needs).[9] A well-designed SCED generates internally valid, high-level evidence.[9] SCEDs are ideal for evaluating interventions in participants with severe impairments and high support needs because they require smaller samples than group-based designs and therefore permit: (1) the allocation of time and expertise required to safely supervise participants with severe primary impairments and multiple comorbidities who are at increased risk of serious adverse events; (2) the provision of the personalised assistance required to mitigate the increased time cost associated with training; and (3) the methodological freedom to individualise training type, duration and intensity without compromising experimental control.

We recognise that the small sample sizes limit SCED external validity, and thus SCEDs should complement, not replace, RCTs. However, wider adoption of SCEDs and standardised, detailed clinical profiling will facilitate development of an evidence-base in an otherwise under-researched population, and ultimately permit an evidence synthesis or meta-analysis to offset the small samples in individual studies.

A recently published SCED investigated the therapeutic benefit of performance-focused swimming training in three previously inactive adolescents with CP-HSN.[10] The SCED permitted: allocation of time and expertise required to assist and supervise participants with severe primary impairments and multiple comorbidities who were at increased risk of serious adverse events (eg, drowning, aspiration); provided the methodological freedom to individualise training parameters without compromising internal validity; and provided direct evidence that initial improvements in gross motor function were lost when training ceased and recovered when training resumed.[10] It also permitted a 30-month follow-up evaluating participant adherence—which remained at 100%—and provided direct evidence that intervention benefits were maintained over time. It is difficult to envisage how these data and the insights they provide could be generated by an RCT.

CONCLUSIONS

SCEDs provide a rigorous and effective means of enhancing both equity and precision of P-SEM research evidence in clinically complex populations that are under represented in P-SEM research such as people with CP-HSN. Other patient groups in which SCEDs can be usefully applied to enhance equity and precision of P-SEM evidence include people with high support needs who have spinal cord injury, traumatic brain injury and multiple sclerosis.

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