Finding the balance

At the core of speech and language therapy practice are clinical decisions about which intervention is appropriate for each person given their goals and pattern of strengths and needs. Evidence-based practice (EBP) guidelines encourage us to look to the research literature to support this clinical decision-making. These same guidelines also provide suggestions on which kinds of research provide the 'best' evidence. Until recently, they have overwhelmingly promoted the view that the 'best' evidence comes from (high-quality) randomised controlled trials (RCTs) and the highest level of evidence has been accorded to systematic reviews and meta-analyses of RCTs.

However, there have long been concerns that RCTs may not always provide all the answers (Howard, 2016). Here, we make the case for valuing evidence from well-controlled single case designs. To quote one SLT responding to a survey (reported alongside fuller exploration of RCTs and single case experimental designs in Best et al, 2019):

> *"I think SLTs should take more confidence in the single case study design as a useful research tool. I find this research evidence more informative as it translates more readily into clinical practice."*

Importantly, and inherent in their design, the outcomes of RCTs apply to groups rather than individuals. Even if an intervention shows a significant benefit in an RCT, this is only true on average: not every individual in the group may benefit. Hence, while another 'acceptably similar' group may also benefit on average, an individual client cannot be guaranteed to improve (e.g., Cohen et al, 2004). This is problematic particularly when there is heterogeneity in the disorder, as in many health conditions including communication disorders. For example, in research into trigger profiles for migraine, analysis of individual profiles (*n*-of-1 analysis) rather than the group average revealed a high degree of inter-individual heterogeneity: the percentage of patients with unique trigger profiles was high (74%) and therefore the group average profile was not applicable to the *majority* of individuals with migraine (Perris et al, 2016).

Returning to intervention, because RCTs determine treatment effectiveness by comparing (treated, untreated and/or differently treated) groups, they are less able to determine which factors influence the extent to which an individual will benefit. This is because changes in the treated group will be a combination of specific treatment effects and other non-specific effects, such as practice, placebo effects, development, or spontaneous recovery. Hence, attempts to identify participant characteristics that may moderate intervention success in an RCT is limited because individuals are likely to beinfluenced by

confounding factors to different degrees. Furthermore, the comparison/control conditions used influence the conclusions that can be drawn.

Within medicine, these problems in identifying individual patterns from RCTs have led to the suggestion that to determine the effectiveness of intervention for an individual, single case investigations are required. Moreover, it has now been recognised that evidence of the highest strength can be obtained at the level of the individual with appropriate methodology. The Oxford Centre for Evidence Based Medicine (2011) places systematic reviews of randomised trials or *n*-of-1 trials at the top of the hierarchy of evidence for an intervention's effectiveness. Additionally, the new Medical Research Council framework for developing and evaluating complex interventions (Skivington et al, 2021) highlights the importance of going beyond whether an intervention works to consider wider questions including the context and resources.

In our field, there are many well-conducted intervention studies that use single case experimental designs (SCEDs). We use the term SCED broadly to encompass different designs that maintain experimental control in a single case or case series intervention study (e.g., Howard et al, 2015; Rvachew and Matthews, 2017).

Given the characteristics of RCTs outlined, particularly the lack of ability to predict an outcome for an individual, it is unclear why SCEDs remain overlooked as potential sources of high-quality evidence. One problem is that there are many single case reports which cannot provide evidence that changes in performance are due to the intervention. These include observational case studies (i.e., clinical descriptions or case reports) that do not include methods to measure, or control for, change of performance over time that is not treatment-related (e.g., due to spontaneous recovery or development) (Perdices and Tate, 2009).

There are, however, clear guidelines regarding SCEDs that enable investigators to be confident that changes are due to the intervention (e.g., Franklin, 1997; Howard et al, 2015; Rvachew and Matthews, 2017). Designs include for example, crossover, multiple-baseline, and withdrawal/reversal. Importantly, they monitor changes in performance across structured intervention and no-intervention phases and treated and untreated items or tasks. While there is no control *group*, each participant acts as their own control, with replication within participant. The designs enable us to establish a causal relationship between intervention and response in each participant (Rvachew and Mathews, 2017). Furthermore, results from SCEDs can be combined to provide insights as informative as those from systematic reviews of RCTs (e.g., Sze et al, 2020) and can be included in meta-analyses (e.g., Roberts and Kaiser, 2011).

Findings from SCEDs are sometimes criticised on the grounds of limited generalisability to other individuals. However, they can enable exploration beyond whether the treatment works to *why* it works. Specific hypotheses can be made regarding the 'active ingredients' of an intervention that can make it effective only for some individuals. Research can ask what are the characteristics of the client and the treatment itself that are essential for efficacy? These hypotheses can then be tested by replication, with different

intervention within the same person (testing active ingredients of the task) and/or with the same intervention in different clients.

In a survey of UK SLTs (reported in Best et al, 2019) respondents valued research from both designs, but found SCEDs significantly more useful than RCTs for guiding client management and planning specific therapy. However, SLTs also noted that research employing RCTs was particularly useful for securing funding and was influential with commissioners of services.

Table 1 summarises and contrasts some key strengths and weaknesses of RCTs and SCEDs. Therapists should always be aware that peer review is not infallible. Poor-quality studies of both types still appear in the literature and claims that go beyond the strength of the data or design are common.

Table 1: A comparison of RCT and SCED research designs for evaluating the effectiveness of interventions with people with communication disorders (adapted from Best et al, 2019).

Research design issue	Randomised controlled trials - group RCT	Experimentally controlled case series/single case designs - SCEDs
Scientific/clinical understanding	Widely accepted as 'gold standard'. Range of well-established designs and analyses.	Less well understood. Range of designs and analyses available.
Experimental control	Established through a control group. The similarity between control and intervention conditions affects the conclusions that can be drawn.	Established in several ways, e.g., baseline testing, control items, modalities or tasks. Participants act as their own control.
Comparison of effectiveness between interventions	Comparisons are usually between groups, e.g., intervention group type 1, intervention group type 2 and control group.	Comparisons are made across phases of treatment/no treatment/different treatments, and/or treated/untreated items, within an individual or across a case series, allowing specific conclusions about appropriate intervention(s) for an individual.
Applicability to individual participants	If a significant difference is found, this is in the group average; the intervention may not benefit all those who are treated.	Findings are applicable on a case-by-case basis. Results are analysed for each participant separately.
Detailed profiling	Usually not possible, given group size necessary for a fully powered study and cost.	Use of in-depth assessment to profile participants communication needs and strengths is feasible and common.
Generalisation to others	Findings are generalisable to others who meet the entry criteria for the study. As the findings are based on group means, they may not generalise to a specific individual.	Findings apply to those included in the study. Replication may extend the results to others with similar communication profiles.
Heterogeneity	Variability inherent in communication disorders means identifying relevant variables and matching groups can be problematic. Heterogeneity may be statistically addressed by using large samples.	Variability inherent in communication disorders and in intervention outcomes can be exploited to allow conclusions linking outcome to the nature of individual profiles across case series of intervention studies.
Selective reporting and publication bias	Some variability: null outcomes have been reported. Risk of bias scales increasingly used.	Potential for vulnerability in SCED studies. However, null findings for an approach or individual often reported in the context of a comparison between approaches/individuals.
Random assignment	Participants are randomly assigned to different groups, a requirement for good RCTs.	Randomisation is important and achieved in various ways, e.g., items may be randomly assigned to intervention and control conditions, intervention order may be randomly assigned.

So, where does this leave us? A clinically informative research question is 'what works for whom and under which conditions?' We suggest that a SCED-case series is well able to answer this through systematic investigation of the effectiveness of treatment, and variability in outcomes, across a series of individuals, with detailed profiling of clients' strengths and difficulties. This research should be considered a key source of high-quality evidence in its own right, rather than a stepping-stone on the way to an RCT. SCEDs can provide a window on causal relationships between intervention and response in each participant.

To be clear, RCT research is, and will continue to be, crucial. Well-conducted RCTs can provide useful evidence and this design will certainly continue to be influential for policy and practice. There are now studies that use RCTs with designs that also enable full analysis at the level of the individual (as in design 9 in Ebbels, 2017, e.g., Best et al, 2021; Smith-Lock et al, 2015;).

Our aim is to question the automatic supremacy of RCTs and encourage researchers, clinicians, policy makers and funders to value other approaches to establishing clinical efficacy, especially well-designed SCEDs. The clinical decision making of SLTs is multifaceted and EBP has multiple pillars, including client preference and clinical expertise. When considering published evidence on interventions as part of EBP, including the results from good quality SCEDs will maximise clinical impact.

To benefit adults and children with communication needs, increasing emphasis needs to be placed on working together to devise research questions and carry out jointly constructed studies embracing the complexity this entails. As we move beyond 'does intervention work?' and the limitations of RCTs become more widely understood and acknowledged, we need to be equipped with a range of evidence that is clinically informative including building a shared understanding of how positive, intervention-induced change can occur and which intervention components are key to that change for those with different communication profiles.

Wendy Best, Professor of Communication Science and Language Therapy, UCLw.best@ucl.ac.uk

Wei Ping Sze, PhD candidate UCL, Lecturer (part-time, National University of Singapore), weipingsze@gmail.com

Jennie Grassly, PhD, Lecturer in Speech and Language Therapy, UCL
j.grassly@ucl.ac.uk

Lyndsey Nickels, Professor, School of Psychological Sciences, Macquarie University, Sydney
Lyndsey.nickels@mq.edu.au@LyndseyNickels