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A stepped wedge design for testing an effect of intranasal insulin on cognitive development of children with *Phelan-McDermid* syndrome: A comparison of different designs

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This paper compares the power of the parallel group design, the matched-pairs design, and several options for the stepped wedge design and delayed start design for testing a possible effect of intranasal insulin with respect to placebo on developmental growth of children with a rare disorder like Phelan-McDermid syndrome. A subject-specific mixed effects model for the primary outcome developmental age in a longitudinal setting with five time points was assumed. Monte Carlo simulation studies with small sample sizes were applied since the rare disorder prohibits large trials. The stepped wedge designs, which were initially preferred for ethical reasons, appear to be competitive in power to other designs and were in some settings even the best. The assumed statistical model also demonstrates that all the designs can be viewed as a delayed treatment design. Under the formulated statistical conditions, our results show that the stepped wedge design is appropriate for randomized controlled trials on developmental growth with small numbers of participants.

Biography

Edwin R Van Den Heuvel performed a PhD in Mathematical Statistics at the University of Amsterdam in 1996. He changed his career to industrial statistics at IBIS UvA, n consultancy institute at the University of Amsterdam. In 2002, he became Director of the Statistical Department of the pharmaceutical company MSD. He has received a fellowship at the Eindhoven University of Technology (TU/e) and a part-time position as Professor in Statistics for Life Sciences at the University of Groningen. In 2010, he became a Professor in Medical Statistics at the University Medical Center Groningen, but holds now a Chair in Statistics at the Mathematics Department of Eindhoven University of Technology.

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