COMMENTARY & PERSPECTIVE

Commentary on an article by Pamela Thomason, BPhy, MPT, et al.: "Single-Event Multilevel Surgery in Children with Spastic Diplegia. A Pilot Randomized Controlled Trial"

By Mark F. Abel, MD, and Diane L. Damiano, PhD, PT

The management of cerebral palsy has evolved substantially over the last seventy years. Motion analysis laboratories have played a pivotal role in this evolution by providing a means with which to study the interrelationship of limb segments for the purpose of devising treatment plans and evaluating results after treatment¹⁻³. Advances in tone management including selective dorsal rhizotomy and medications such as baclofen and botulinum toxin^{4,5}, and the advent of functionally based physical therapies replacing traditional neurodevelopmental approaches⁶ were other important contributions. The World Health Organization (WHO) framework, the International Classification of Functioning, Disability and Health (ICF), and the concurrent validation of outcome instruments for classifying and assessing children with chronic motor disabilities such as cerebral palsy were indispensable to the design of clinically relevant and more objective outcome studies⁷. The widespread adoption of single-event multilevel surgery as the mainstay orthopaedic surgical approach for gait abnormalities in patients with cerebral palsy was also a part of this evolution. However, single-event multilevel surgery is only one component of the multifaceted management approach for patients with cerebral palsy. Nevertheless, Thomason et al. are to be congratulated for providing the first published randomized controlled trial to evaluate the outcome of single-event multilevel surgery with use of outcome measures that assess several domains within the ICF framework, including impairments of body structure and functional capabilities.

The study confirms what other prospective cohort studies of the lower extremity have shown—i.e., that single-event multilevel surgery results in short-term (twelve-month) improvement in gait alignment while its impact on functional capacity is negligible or minimal as measured with the Gross Motor Function Measure (GMFM)⁸⁻¹⁰. The results showed a larger effect on the two kinematic outcome measures and a lesser effect on functional outcomes at twelve months as compared with the effects found in most other studies. Both the Gait Profile Score (GPS) and the Gillette Gait Index (GGI) are largely calculated on the basis of the degree of differences in three-dimensional limb position as compared with a normative database. The most likely explanation for the somewhat different pattern of change in this study is the high prevalence of rotational realignment procedures (done in ten of eleven subjects), which contributes to more dramatic changes in kinematic values compared with those resulting from muscle-tendon lengthening procedures alone. This difference may also account for the extended surgical recovery with the functional changes not appearing until twenty-four months after baseline. However, the result at twenty-four months postoperatively was an observational result; the authors did not perform a controlled comparison.

The comparison of effect sizes in this trial with those from previous studies supports recent challenges to the long-held assumption that, compared with randomized controlled trials, observational studies typically exaggerate treatment effects. A report in *The New England Journal of Medicine* by Concato and colleagues¹¹ compared results regarding five clinical topics between randomized controlled trials and observational studies and showed that observational trials do not systematically overestimate the magnitude of effects. In fact, when compared with randomized controlled trials, the observational studies were found to have less variable results and to be less likely to produce contradictory conclusions. The major advantage of randomized controlled trials is that they optimize internal validity or the confidence that the differences between groups are due to the intervention rather than to differences in patient characteristics. In the randomized controlled trial by Thomason et al., participant age, sex, and size appeared similar across groups. However, scores for characteristics that would have influenced the primary outcomes such as baseline function, as measured with the Child Health Questionnaire (CHQ), the GMFM, and the Gross Motor Function Classification System (GMFCS), were lower in the surgical group. Given the small sample size, this study could be criticized for not being generalizable were it not for the previous prospective cohort studies mentioned above.

Accepting that the net effect of single-event multilevel surgery on the gait function of patients with cerebral palsy is positive, practitioners also recognize that the precise combination of surgical procedures chosen to address the gait problems exhibited by these patients can vary by center and surgeon. Motion data provide an objective assessment of dynamic gait function and limb alignment but do not yield a precise surgical prescription, and several studies have shown discordance among surgery decisions made by surgeons on the basis of the same gait data^{12,13}. This pilot study, with its small sample sizes of eleven in the surgical group and eight in the control

group, fails to provide new insights into the precise indications for a particular muscle-tendon procedure or the incremental value of an osseous surgical procedure for torsional or angular deformity. Thus, the hypothetical concept that realignment of osseous levers is important for functional gain remains unproven. Furthermore, technical aspects of the surgical techniques vary widely, and no study has provided insight into how much length is adequate for a particular muscle-tendon unit or if precise length measurements are important for surgical decision-making. By designing the study to move the control group to surgery after twelve months, the authors missed an opportunity to test, with a more robust study design, the hypothesis that the gait pattern of patients with cerebral palsy deteriorates over time as suggested by prior retrospective reports^{14,15}. In the current study, the “control” group, which actually participated in a physical therapy strengthening intervention, showed improved “physical function” scores, comparable with those in the surgical group, on the CHQ at the twelve-month evaluation despite a lack of improvement in kinematics. The divergent changes in functional and kinematic results in the control group illustrate the complexity of the relationship between impairment-based (kinematic) and functional outcomes, the latter of which should be the paramount goal of treatment. Also, the authors missed an opportunity to evaluate the consistency of the surgical effects by not reporting the changes experienced in the control group twelve months after they underwent single-event multilevel surgery.

Finally, the goal of research should be to produce the best and most relevant evidence to guide clinical practice for optimal patient care. Randomized controlled trials are paramount for establishing the efficacy of a specific intervention in a well-defined patient population, but as Concato et al. concluded, “The popular belief that only randomized, controlled trials produce trustworthy results and that all observational studies are misleading does a disservice to patient care, clinical investigation, and the education of health care professionals.”¹¹ A recent editorial by one of the authors of the current trial echoes emerging views on the most appropriate research designs for complex clinical questions in diverse patient populations such as improving motor patterns in patients with cerebral palsy¹⁶. Graham contended that the majority of surgical interventions in this population are “not amenable to randomized controlled trials.” Instead, he made a plea for the conduct of large-scale, multicenter observational studies to compare the effectiveness of interventions in the actual practice settings. These “practice-based evidence” methodologies involve use of advanced statistical techniques on large prospectively collected comprehensive data sets to identify which treatment approaches and/or patient characteristics are associated with the best and worst outcomes. These techniques have already been employed successfully in multiple adult rehabilitation populations such as patients with stroke and traumatic brain injury, and there has been a recent large-scale study examining outcomes of joint replacement surgery¹⁷. While it is important to validate the need for surgery, albeit in a general sense as this randomized controlled trial clearly has done, we need to be far more precise in our decisions of which surgical procedures should be done and who should undergo them.

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