

Gimeno, H., Polatajko, H. J., Cornelius, V., Lin, J.-P., & Brown, R. G. (2018). Protocol for *N*-of-1 trials proof of concept for rehabilitation of childhood-onset dystonia: Study 1. *Canadian Journal of Occupational Therapy*, 85, 242–255. doi:10.1177/0008417417707532

Appendix A: Outcome measures description

Secondary Outcomes

Pediatric Evaluation of Disability Inventory Computer Adaptive Test (PEDI-CAT; Haley et al., 2011). The PEDI-CAT is a self-report questionnaire administered via tablet computer completed by the child's carer. The test captures the child's abilities and the need for caregiver assistance in three domains: daily activities, mobility, and social/cognitive. A fourth domain, responsibility, measures the extent to which the caregiver or child takes responsibility for managing complex, multi-step life tasks.

Adaptive Behaviour Assessment System (ABAS-II; Harrison & Oakland, 2008). The ABAS-II is a parent-completed questionnaire that assesses the frequency with which the child performs adaptive skills in 10 areas. It provides an overall score of adaptive functioning (General Adaptive Composite) and subscores for conceptual skills (communication, functional academics, and self-direction), social skills (leisure and social), and practical skills (community use, home/school living, self-care, health and safety, and work).

Behavior Rating Inventory of Executive Function (BRIEF; Gioia, Isquith, Guy, & Kenworthy, 2000). This parent-reported questionnaire assesses executive functions in everyday life. The BRIEF consists of 8 subscales: initiate, working memory, plan/organize, organization of materials, monitor, inhibit, emotional control, and shift. The first five subscales form the Behavioural Regulation Index (BRI), and the last three subscales form the Metacognition Index (MCI). The combination of the two indices' scores form the overall Global Executive Composite (GEC).

Spence Children's Anxiety Scale (Spence, 1998): This self-report scale is completed by the child with a separate version completed by the parent. The scale assesses the frequency of anxiety symptoms in six domains: generalized anxiety, panic/agoraphobia, social phobia, separation anxiety, obsessive compulsive disorder, and physical injury fears. Items for these domains are allocated randomly within the scale.

Self-Efficacy Gauge Measure–Pediatrics (SEG-P). The SEG-P was developed for

specifically for occupational therapy. The child rates her/his confidence to do daily activities without assistance on a 10-point scale.

EQ-5D (Herdman et al., 2011). This measure of health-related quality of life, developed by the EuroQol group, is completed by the child using age-appropriate versions: EQ-5D-y (6–12 years of age), EQ-5D-5L (12 - 18 years) and EQ-5D-3L (over 18 years). The scale assesses the degree of problems experienced in five dimensions: mobility, self-care, usual activities, pain/discomfort, and anxiety/depression. In addition, a EuroQol Visual Analogue Scale (EQ-VAS) provides an overall self-assessment of health state from “best imaginable health state” (score = 100) to “worst imaginable health state” (score = 0).

Baseline Assessments for Treatment Planning

Canadian Occupational Performance Measure (COPM; Law et al., 1990). The COPM is a self-reported outcome measure completed through structured interview with the child or young person (and/or the carer) at baseline. This measure helps to engage the child and family and provides information useful in focusing the intervention sessions.

Goal Attainment Scale (GAS; Kiresuk, Smith, & Cardillo, 1994). The GAS is a method of scoring the extent to which a child’s goals are achieved with intervention. A goal is established at baseline using the top 5 concerns identified by the child using the COPM.

Baseline Assessment of Staging of Functional Motor Ability

Two measures will be used to characterize the child’s functional motor ability: Gross Motor Function Classification System (GMFCS; Palisano et al., 1997) and the Manual Ability Classification System (MACS; Eliasson et al., 2006). For this study, the information for the scales will be obtained from medical records or by interview with the child and/or parents. These ordinal scales with five levels (I–V) have been validated for use with children with cerebral palsy (CP). The levels provide information about limitations in daily life with higher scores implying more functional difficulties. Higher levels denote more severe difficulties with motor abilities. The use of these scales with children with hyperkinetic movement disorders other than CP provides a clinically useful functional profile (Elze et al., 2016).

GMFCS (Palisano et al., 1997). This scale describes self-initiated gross motor function with distinction between the levels based on differences in functional abilities for independent sitting and walking and the need for assistive technology or wheeled mobility. For this study, classification will be done by the principal investigator using available data from the medical records and through discussion with parents as per GMFCS protocol.

MACS (Eliasson et al., 2006). This scale classifies how children and young people with CP handle objects in everyday life with differences between the levels based on the child's self-initiated ability and the need for assistance or adaptations to perform daily activities. The classification will require the involvement of the children or families in the discussion of the classification as per MACS protocol.

Other Demographic and Clinical Measures

The data for these measures will be obtained from the medical records or interview with the child and/or parent. Demographic measures will be used for descriptive purposes. A range of clinical measures will be used to describe the aetiologic diagnosis and motor phenotype. These will be described by a consultant paediatric neurologist specialized in paediatric movement disorders using clinical observations and a neurological examination. The consultant neurologist will use a motor phenotype characterization checklist based on Sanger's definition and classification of hyperkinetic movement disorders (Sanger et al., 2010). This is a pragmatic solution to characterize a participant's movement disorder that is feasible within the clinical and research context. As no single measure is available to distinguish between all the different hyperkinetic movement disorders that might co-exist, this motor phenotype characterization is possibly the best compromise to inadequate available measures. Brain imaging will be used to describe structural neurological damage. Cognitive measures will be used to define the presence and severity of overall intellectual function and executive function.

Demographic measures. Age and gender.

Behavioural Assessment of Executive Syndrome in Children (BADS-C; Norris & Tate, 2000). This battery of tests evaluates executive functions including inhibitory control, planning, problem solving, monitoring behaviour, and cognitive flexibility.

Burke-Fahn-Marsden Dystonia Rating Scale (BFMDRS; Burke et al., 1985). The BFMDRS Movement scale will be used to evaluate dystonia severity following the published protocol. Dystonia severity is rated at rest in nine body regions and with provoking factors such as speaking, writing, and walking. An experienced occupational therapist or physiotherapist will videotape and assess dystonia severity using the BFMDRS-M. The videos will then be randomly presented for blind rating by experienced clinicians.

Dyskinesia Impairment Scale (DIS; Monbaliu et al., 2012). This scale includes two separate measures to assess dystonia and choreoathetosis duration and amplitude. It assesses 12 body regions with a distinction between proximal and distal regions of the limbs. Duration refers

to amount of time whereas the amplitude refers to the range of motion of the involuntary movements. The DIS scores body regions at rest and during two activities (action). The principal investigator will complete the assessments.

Neuroradiological classification. The Magnetic Resonance Imaging (MRI) completed prior to the deep brain stimulation surgery will be used for classification of neuroradiological findings using a semi-quantitative scale for classification of structural brain MRI (Fiori et al., 2014). Two experienced neurologists will complete the classification and results compared for agreement. If agreement is not reached, the MRIs will be taken to a third neurologist.

Wechsler Intelligence Scale for Children (WISC-IV; Wechsler, 2004). A clinical psychologist with experience in paediatric movement disorders will assess cognitive ability using the WISC-IV. Typically, the information will be obtained from the medical records unless the most recent assessment was completed over 12 months ago.

References

- Burke, R. E., Fahn, S., Marsden, D., Bressman, S., Moskowitz, C., & Friedman, J. (1985). Validity and reliability of a rating scale for the primary torsion dystonias. *Neurology*, 35, 73–77.
- Eliasson, A. C., Krumlinde-Sundholm, L., Rosblad, B., Beckung, E., Arner, M., Ohrvall, A. M., & Rosenbaum, P. (2006). The Manual Ability Classification System (MACS) for children with cerebral palsy: Scale development and evidence of validity and reliability. *Developmental Medicine & Child Neurology*, 48, 549–554. doi:10.1017/S0012162206001162
- Elze, M. C., Gimeno, H., Tustin, K., Baker, L., Lumsden, D. E., Hutton, J. L., & Lin, J. P. (2016). Burke-Fahn-Marsden dystonia severity, gross motor, manual ability, and communication function classification scales in childhood hyperkinetic movement disorders including cerebral palsy: A “Rosetta Stone” study. *Developmental Medicine & Child Neurology*, 58, 145–153. doi:10.1111/dmcn.12965
- Gioia, G. A., Isquith, P. K., Guy, S. C., & Kenworthy, L. (2000). *Behavior Rating Inventory of Executive Function (BRIEF) manual*. Lutz, FL: Psychological Assessment Resources.
- Haley, S. M., Coster, W. J., Dumas, H. M., Fragala-Pinkham, M. A., Kramer, J., Ni, P., . . . Ludlow, L. H. (2011). Accuracy and precision of the Pediatric Evaluation of Disability

- Inventory computer-adaptive tests (PEDI-CAT). *Developmental Medicine & Child Neurology*, 53, 1100–1106. doi:10.1111/j.1469-8749.2011.04107.x
- Harrison, P., & Oakland, T. (2008). *Adaptive Behavior Assessment System* (2nd ed.). New York, NY: Elsevier.
- Herdman, M., Gudex, C., Lloyd, A., Janssen, M., Kind, P., Parkin, D., . . . Badia, X. (2011). Development and preliminary testing of the new five-level version of EQ-5D (EQ-5D-5 L). *Qualitative Life Research*, 20, 1727–1736. doi:10.1007/s11136-011-9903-x
- Kiresuk, T. J., Smith, A., & Cardillo, J. E. (Eds.). (1994). *Goal Attainment Scaling: Applications, theory, and measurement*. Hillsdale, NJ: Lawrence Erlbaum. .
- Law, M., Baptiste, S., McColl, M. A., Opzoomer, A., Polatajko, H., & Pollock, N. (2014). *Canadian Occupational Performance Measure* (5th ed.). Ottawa, ON: CAOT Publications ACE.
- Monbaliu, E., Ortibus, E. L. S., de Cat, J. O. S., Dan, B., Heyrman, L., Prinzie, P., . . . Feys, H. (2012). The Dyskinesia Impairment Scale: A new instrument to measure dystonia and choreoathetosis in dyskinetic cerebral palsy. *Developmental Medicine & Child Neurology*, 54, 278–283. doi:10.1111/j.1469-8749.2011.04209.x
- Norris, G., & Tate, R. L. (2000). The Behavioural Assessment of the Dysexecutive Syndrome (BADS): Ecological, concurrent and construct validity. *Neuropsychological Rehabilitation*, 10, 33–45. doi:10.1080/096020100389282
- Palisano, R. J., Rosenbaum, P., Walter, S. D., Russell, D. J., Wood, E., & Galuppi, B. (1997). Development and reliability of a system to classify gross motor function in children with cerebral palsy. *Developmental Medicine & Child Neurology*, 39, 214–223. doi:10.1111/j.1469-8749.1997.tb07414.x
- Sanger, T. D., Chen, D., Fehlings, D. L., Hallett, M., Lang, A. E., Mink, J. W., . . . Valero-Cuevas, F. (2010). Definition and classification of hyperkinetic movements in childhood. *Movement Disorders*, 25, 1538–1549. doi:10.1002/mds.23088
- Spence, S. H. (1998). A measure of anxiety symptoms among children. *Behavior Research and Therapy*, 36, 545–566.
- Wechsler, D. (2004). *Wechsler Intelligence Scale for Children* (4th ed.). London, UK: Pearson Education.