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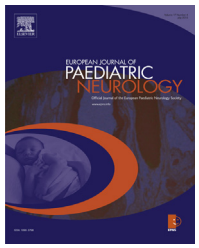

Original article

Evaluation of functional goal outcomes using the Canadian Occupational Performance Measure (COPM) following Deep Brain Stimulation (DBS) in childhood dystonia

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ABSTRACT

Purpose: To evaluate the functional goal-directed outcomes of Deep Brain Stimulation (DBS) in childhood dystonia according to aetiology and to explore relationship with a traditional impairment-based measure.

Method: This is a prospective case series study involving thirty children with dystonia with a 1-year follow-up post-DBS. The Canadian Occupational Performance Measure (COPM) and Burke-Fahn-Marsden Dystonia Rating Scale (BFMDRS) were used as primary outcome measures. Results were analysed based on aetiology in 3 groups: 1. primary/primary plus dystonia; 2. secondary dystonia–cerebral palsy (CP); 3. secondary dystonia–non-CP group. Correlation between functional outcome using COPM and dystonia improvement as captured by BFMDRS was measured.

Results: All groups demonstrated significant improvement in individualised goal attainment, measured with the COPM, at 1-year post-DBS. The secondary dystonia-CP group also achieved significant improvement at 6 months for performance and satisfaction scores. In the majority of secondary dystonias, the BFMDRS failed to demonstrate significant improvement. A linear correlation between change in BFMDRS and COPM scores was observed when the entire cohort was analysed.

Interpretation/conclusions: DBS improved functional performance, independently of the dystonic phenotype. Improvements in individualized COPM functional goal areas were seen in the absence of significant changes in BFMDRS scores, highlighting the relative insensitivity of impairment scales in this patient group.

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1. Background

Neuromodulation via Deep Brain Stimulation (DBS) is an evolving therapeutic option for childhood refractory dystonia.[1] The mechanism of action in dystonia remains unclear but alteration of neuronal plasticity may play an important role.[2,3]

Dystonia is characterised by patterned, directional, and often sustained muscle contractions that produce abnormal postures and/or repetitive movements.[4] Classification of dystonia is based on age of onset, distribution and nature of clinical features and cause.[5,6] Childhood dystonia is characterised by its heterogeneity[7,8] with secondary dystonias more common than primary dystonias.[7]

The efficacy of DBS in idiopathic primary dystonias is well-established,[9,10] but the role of DBS in the management of secondary dystonias remains poorly understood.[11–13] The majority of reported outcomes for DBS in childhood dystonia have used dystonia rating scales such as the Burke-Fahn-Marsden Dystonia Rating Scale (BFMDRS).[14–18] Marks et al., 2011 report a 24% improvement in BFMDRS in 14 children with dystonic cerebral palsy (CP) which also included a group of DBS 'non-responders.'[14] A meta-analysis of 68 children with dystonic CP drawn from the literature has also reported a 23.6% mean BFMDRS improvement after DBS.[19] However, in one of the larger studies, children with secondary dystonia, particularly cerebral palsy (CP), exhibited little change on dystonia rating scales following DBS despite positive subjective parental feedback.[15] A number of other groups report similar trends of smaller changes in BFMDRS after DBS in secondary dystonias.[12,18,20,21] We have previously demonstrated improvement in functional goals set pre-operatively, using a range of standardised outcome measures and goal-setting tools, in 6 cases of severe secondary dystonia in whom the BFMDRS indicated apparent non-response to DBS,[8] highlighting the limitations of impairment scales such as the BFMDRS if used in isolation and emphasising the need to measure beyond impairment and across the domains of the International Classification of Functioning, Disability and Health (ICF).[22]

We have recently used the Canadian Occupational Performance Measure (COPM)[23,24] to describe the impact of dystonia on daily life by identifying the top 5 functional concerns in children and young people with dystonia.[8] We found no differences in the type of concerns identified when groups were analysed according to aetiology or level of function, suggesting that dystonia affected similar areas of activity and participation for all individuals, independently of its cause or severity.

The COPM has been used as the primary outcome measure when evaluating therapeutic and medical interventions.[25–27] Most studies in the paediatric CP literature have focused on children with primarily spastic phenotypes with few studies solely focusing on functional outcomes following interventions in children with dystonia including dystonic-CP.[13,28] The COPM has been used to demonstrate changes in small cohorts of children with dystonia following surgical intervention such as continuous intrathecal baclofen (ITB) infusion,[26,29] though no individualised scores were provided and sample sizes have been small.

The World Health Organisation advocates client-centred outcome measures. These are largely missing in outcomes research involving paediatric dystonia, particularly when evaluating neurosurgical interventions such as DBS. A greater understanding of the concordance between functional and impairment-based measures, such as BFMDRS is needed, particularly when reporting cases of paediatric secondary dystonia.[8,18]

This study aims to report the extent to which pallidal DBS in paediatric dystonia can meaningfully address the functional concerns raised by children and families. We hypothesised that DBS would improve functional outcomes in childhood movement disorders, with greater improvement anticipated in primary dystonia subjects.

2. Method

2.1. *Participants*

Data obtained from a prospective audit of cases undergoing DBS (from 2008 to 2012) and who had participated in routine, formal pre-operative goal-setting. Because the study was routine clinical practice, ethics approval was not required and consent was neither required nor obtained.

Study participants included the parents or carers of children and young people (aged 3–17 years) with dystonic movement disorders undergoing DBS surgery with a minimum of 1-year follow-up in whom formal goal evaluation was established using the COPM.

2.2. *Measures*

2.2.1. *Classification of ability*

The Gross Motor Function Classification System (GMFCS)[30] and Manual Ability Classification System (MACS)[31] were used to classify levels of gross motor and manual ability function respectively. While these ordinal scales have been validated for use in CP, "equivalent" GMFCS/MACS scores were applied to non-CP cases in order to capture information about functional performance, regardless of aetiology as previously outlined.[8,13,18]

2.2.2. *Functional measures*

Functional concerns prior to DBS were identified using the COPM. Each concern was rated on a scale of 1–10 in terms of importance (1 = least important, 10 = most important); perception of current performance (1 = not able to do it, 10 = able to do it extremely well); and satisfaction with current performance (1 = not satisfied at all, 10 = extremely satisfied). Primary goals for surgery were established by identifying the top 5 concerns as previously described.[8]

2.2.3. *Dystonia rating scale*

The BFMDRS[32] was implemented at baseline and subsequent post-operative reviews. The video assessment protocol was completed by one of the Complex Motor Disorders Service (CMDS) team members, using original guidelines and videoed for subsequent scoring.

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2.3. Procedure

Clinical evaluation was performed pre-operatively and 6 monthly following DBS surgery in an open-label design. Evaluators were not blinded to the patient's treatment status.

2.3.1. Aetiology and severity classification

The cases were classified by aetiology and assigned to dichotomised GMFCS and MACS levels into "more functional" and more "severely impaired" groupings, as previously described.[8] Classification of cases as either primary/primary plus or secondary dystonia was performed prospectively as part of our service clinical protocol. The secondary dystonia group was further sub-classified into CP-related and non-CP-related dystonia.

2.3.2. Functional measure

The COPM has been used to describe functional priorities in children with dystonia[8] and was implemented with parents of a consecutive sample of children attending the paediatric CMDS by one of two therapists (HG & KT) 1-year post-DBS and at 6 months when possible, to determine change in the participants' perceptions.

Response to DBS intervention was determined by evaluating change in the COPM performance (COPM-P) and satisfaction (COPM-S) scores between pre- and post-operative time points. For data analysis, COPM-P&COPM-S scores were averaged (i.e. ratings for each area identified were summed and divided by the number of areas). A difference of 2 points is considered significant.[24]

2.3.3. Dystonia measure

BFMDRS testing was completed as per published guidelines,[32] with movement severity (BFMDRS-M) and disability (BFMDRS-D) scales scored at all 3 time points. Scoring from video was performed by two of three consistent raters with reference to instructions from the original publication. While raters were not blinded to the intervention, previous BFMDRS results were not available at the time of scoring follow-up videos to reduce recall bias. Scores are reported as a percentage change: i.e. pre-operative score minus follow-up score divided by total score and multiplied by 100. A post-operative change of at least 20% has been previously considered to be clinically significant.[33,34] The BFMDRS scores included in this manuscript have previously been reported elsewhere.[18]

2.4. Surgical procedure

Pallidal DBS surgery was performed following a well-established surgical protocol.[35] Electrodes were inserted under stereotactic guidance, bilaterally in 29 cases and unilateral in one case. Further details of surgical technique have been reported elsewhere.[18]

2.5. Data analysis

Results were explored in relation to aetiology based on three groups: 1. primary/primary plus dystonia, 2 .secondary dystonia-CP and 3 .secondary dystonia non-CP group.

Statistical analysis of results pre- and post-DBS was performed using the Statistical Package of Social Science (SPSS version 17.0) (SPSS Inc, Chicago, USA). Kruskal–Wallis testing was used to test the null hypothesis of equivalent median responses across the aetiological groups at baseline, 6 months and 1-year following DBS. The Wilcoxon Signed Ranked Test was used to determine whether mean population ranks differed comparing baseline and post-intervention scores. In all cases a p-value <0.05 was considered statistically significant.

A linear regression analysis was performed to explore the relationship between functional outcome, using the COPM, and dystonia improvement, as captured by the BFMDRS, accounting for age at surgery and duration of symptoms.

3. Results

Clinical characteristics are given in Table 1 and summarised in Table 2. The majority of children were classified as secondary dystonia ($n = 21/30$), with dystonic CP comprising the largest group ($n = 14/30$). The secondary dystonia non-CP group ($n = 7/30$) included metabolic causes ($n = 2/30$), acquired brain injuries ($n = 1/30$) and progressive disorders ($n = 4/30$) such as neurodegeneration with brain iron accumulation (NBIA) ($n = 2/30$). The primary/primary-plus dystonic group ($n = 9/30$) included children with and without known gene mutations: DYT1 positive ($n = 3/30$), DYT1 negative ($n = 3/30$), gene negative early-onset/juvenile dystonia-parkinsonism ($n = 1/30$) and early onset dystonia associated with learning difficulties of unknown cause ($n = 2/30$). One patient had hemiplegic distribution while the rest had generalised dystonia.

Approximately two thirds of the cohort had severe motor impairment on baseline evaluation, with $n = 19/30$ classified as GMFCS IV–V and $n = 20/30$ classified as MACS IV–V. Age at surgery ranged from 3.5 to 18 years. The median age for children with primary dystonias was 13.8 years, but only 9.3 years for secondary dystonias.

No stimulation tolerance was observed in this sample of children.

3.1. COPM data

COPM data available for 22 cases at 6 months and 30 at 1-year post-DBS.

Scores were not normally distributed at any time point and non-parametric testing found no significant difference in COPM scores between the primary or secondary groups at baseline, 6 or 12 months. COPM-P&COPM-S results for the groups are illustrated in Fig. 1(A) and (B). Significant change in COPM-S scores was obtained for the primary dystonia/dystonia-plus group at six months ($p = 0.028$) and one year ($p = 0.008$) but only at 1 year for COPM-P scores (6 months $p = 0.063$, 1 year $p = 0.008$). The secondary dystonia-CP group achieved significant change in both COPM-P (6 months $p = 0.005$, 1 year $p = 0.001$) and COPM-S (6 months $p = 0.005$, 1 year $p = 0.001$). For the non-CP secondary dystonic group, results reached significance only at 1 year for both COPM-P&COPM-S (6 months $p = 0.080$, 1 year $p = 0.0028$).

Individual results in Table 3 show COPM-P&COPM-S scores at baseline and change in scores obtained at subsequent reviews with medians and quartiles in Table 4. The majority of

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Table 1 – Clinical demographics.

Pt	Classification		Diagnosis	Age at surgery	Duration	Gender	GMFCS	MACS
1	Primary/primary plus syndrome	Idiopathic primary dystonia	DYT 1 +ve	14 y 11 m	3	M	2	2
2		Idiopathic primary dystonia	DYT 1 +ve	11 y 7 m	3.75	M	3	1
3		Idiopathic primary dystonia	DYT 1 +ve	13 y 9 m	4	M	4	4
4		Idiopathic primary dystonia	DYT 1 –ve	7 y 4 m	1	F	4	2
5		Idiopathic primary dystonia	DYT 1 –ve	17 y 4 m	13	F	1	2
6		Idiopathic primary dystonia	DYT 1 –ve	12 y 2 m	12	F	1	1
7		Primary plus	Early onset and LD	16 y	14	F	5	5
8		Primary plus	Early onset and LD	10 y	3	F	2	2
9		Primary plus	Early Parkinsonism	14 y 11 m	14	M	4	4
10	Secondary – CP	Secondary CP	CP – 4 limb – Ex Prem	9 y 4 m	9	M	5	5
11		Secondary CP	CP – 4 limb – kernicterus	12 y 3 m	12	M	5	5
12		Secondary CP	CP – 4 limb – kernicterus	5 y 11 m	7.20	M	3	4
13		Secondary CP	CP – 4 limb – Ex preterm	12 y 1 m	10.80	M	4	4
14		Secondary CP	CP. 4 limb. Ex preterm	5 y 4 m	5.40	M	5	5
15		Secondary CP	CP. 4 limb. Ex Preterm	11 y 8 m	12	M	5	5
16		Secondary CP	CP. 4 limb. Ex Preterm	10 y 2 m	10.60	M	5	5
17		Secondary CP	CP. 4 limb. HIE	13 y 7 m	12	M	5	5
18		Secondary CP	CP. 4 limb. HIE	18 y	18.25	F	2	3
19		Secondary CP	CP. 4 limb. Ex preterm	6 y 1 m	6	M	5	5
20		Secondary CP	CP. 4 limb. Ex preterm	6 y 1 m	6	F	5	5
21		Secondary CP	CP. 4 limb. Ex preterm	5 y 3 m	5	M	5	5
22		Secondary CP	CP. 4 limb. Kernicterus	3 y 6 m	3.50	M	5	5
23		Secondary CP	CP. 4 limb. PVL	7 y 11 m	7	M	5	5
24	Secondary-non CP	Secondary non CP (metabolic)	4 limb. Glutaric Aciduria Type 1	11 y 6 m	11	M	5	5
25		Secondary non CP (metabolic)	4 limb. Melathyl-malonic acidaemia.	4 y 8 m	3	M	5	5
26		Secondary non CP (stroke)	Left hemiplegia. Stroke.	13 y 1 m	7	M	1	2
27		Secondary non CP (PKAN)	4 limb. PANK – 2.	7 y	6.20	M	5	4
28		Secondary non CP (NBIA)	4 limb. Neurodegeneration with brain iron accumulation	12 y 10 m	3.90	F	2	2
29		Secondary non CP (Neuromsucular)	4 limb. Evolving dystonia in congenital neuromuscular disorder secondary to a mutation in the LMNA gene	9 y 10 m	2.90	F	3	4
30		Secondary non CP (unknown)	4 limb. Undiagnosed infantile onset dystonic movement disorders (with changes on MRI)	4 y 5 m	3.70	M	4	4

LD = learning difficulties, CP = cerebral palsy, HIE-hypoxic ischaemic encephalopathy, PVL = periventricular leukomalacia, PANK2 = pantotheneate kinase 2, NBIA = neurodegeneration with brain iron accumulation.

Table 2 – Summary of demographic data.

Classification	Number	Gender		Male%	GMFCS		MACS		Age at surgery	Years	
		Male	Female		I–III	IV–V	I–III	IV–V	Median	Minimum	Maximum
Idiopathic primary dystonia	6	3	3	50	4	2	5	1	13	7.3	17.3
Primary Plus	3	1	2	67	1	2	1	2	14.9	10	16
Secondary CP	14	12	2	86	2	12	1	13	8.6	3.5	18
Secondary non CP	7	5	2	72	3	4	2	5	9.8	4.4	13.1
Total	30	21	9	70	10	20	9	21	13.5	10.8	18

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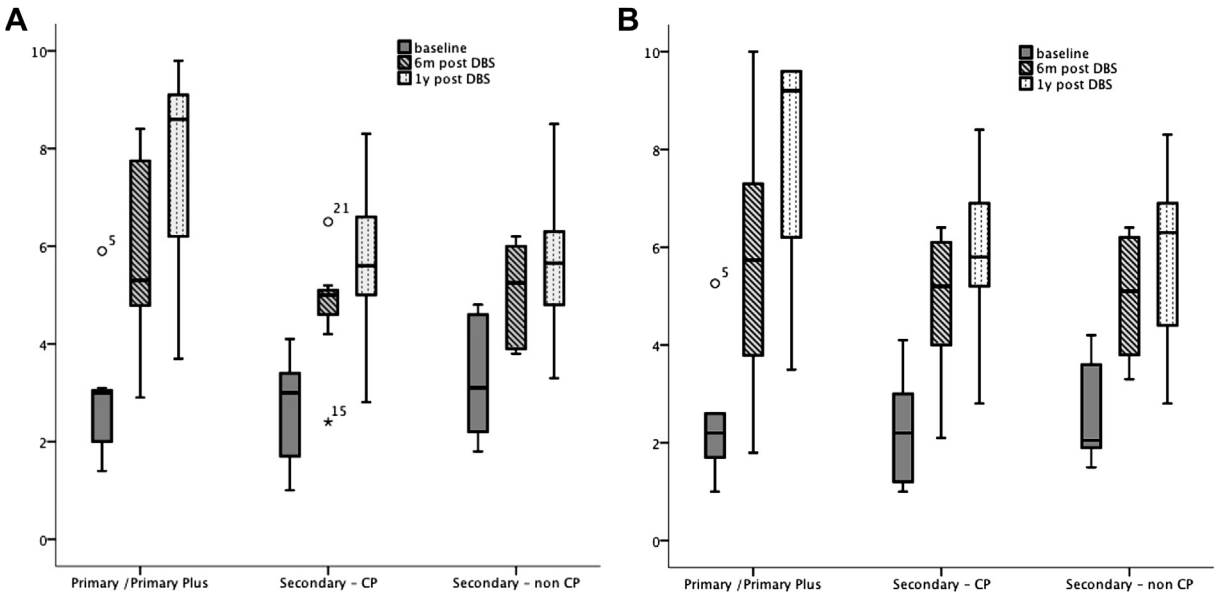

Fig. 1 – (A)COPM – performance scores at baseline, 6 months and 12 months following DBS. (B)COPM – satisfaction scores at baseline, 6 months and 12 months following DBS. Patients have been grouped in primary/primary plus, secondary-Cerebral Palsy and Secondary – non Cerebral Palsy. Improvements in both performance and satisfaction can be seen in the three groups. Statistically signficant change is obtained in the Secondary CP group at 6 and 12 months following DBS (Wilcoxon Signed Rank test, $p < 0.05$). For the primary/primary plus and secondary dystonia-non CP group statistical significant change is observed at 1 year post DBS (Wilcoxon Signed Rank test, $p < 0.05$).

cases demonstrated significant improvement in COPM scores at both 6 months and 1-year post-DBS: COPM-P improvement at 6 months $n = 11/22$ and at 1 year $n = 19/30$; COPM-S likewise improved at 6 months $n = 13/22$, and at 1 year $n = 20/30$.

The primary/primary plus group, $n = 4/8$ demonstrated clinical improvement in performance and satisfaction at 6 months post DBS, increasing to $n = 6/9$ at one year. All but one child with pure primary dystonia achieved clinically significant improvement in functional goal areas, while only one of 3 primary plus dystonia cases achieved clinical significance in COPM goal acquisition.

For the secondary dystonic group, approximately half the cases showed improvement at 6 months (COPM-P improvement $n = 7/15$; COPM-S improvement $n = 8/15$), with a greater proportion showing significant improvement in goal areas at 1-year (COPM-P improvement $n = 13/21$); COPM-S improvement $n = 14/21$). No children demonstrated significant deterioration in COPM scores at any point, despite children with progressive disorders included in the study.

3.2. BFMDRS data

BFMDRS-M scores have previously been reported for children in this cohort[18] and were available for 29 subjects at 6 months and 30 at 1 year. Higher baseline BFMDRS-M scores were found in the secondary compared to primary group. Results including raw data and percentage change are illustrated in Table 3 with a summary of medians and quartiles in Table 4.

Using the >20% change to indicate clinical significance adopted by other groups[33,34] the majority of our secondary dystonia cohort failed to show change in BFMDRS-M scores following DBS. Whilst the majority of primary dystonias showed >20% change (6 of 9 cases at both 6 months and 1 year) only 1 child in the secondary-CP group achieved >20% change at 6 months which was not maintained at 1 year. One child in the secondary-non CP group maintained improvement in BFMDRS-M scores >20% both at 6 months and 1 year.

3.3. Correlation between functional outcomes and impairment measures

At 12 months a significant positive correlation was found between the changes in both COPM-P&COPM-S scores and change in BFMDRS-M scores for the primary/primary-plus group alone (Spearman's correlation coefficient 0.717 and 0.817 respectively, p-values 0.03 and 0.007 respectively). No significant correlations were found for the secondary-CP (Spearman's correlations, coefficient 0.201 and 0.176, p-values 0.511 and 0.565) or secondary-non-CP groups (Spearman's correlation coefficient 0.156 and −0.190, p-values 0.713 and 0.651). When a linear correlation was applied to the whole cohort, even small changes in dystonia as measured by the BFMDRS translated into functional gains. A scatter plot demonstrating the relationships between the changes in these scores is shown in Fig. 2.

Linear regression analysis to model the slope of the relationship between a percentage change in BFMDRS-M and change in COPM-P gave a positive slope of 0.06 (95% CI 0.4–0.8) (ie. a 0.6 increase in COPM-P for a 10% increase in BFMDRS-M) with no significant difference between primary and secondary dystonias. A second regression analysis which omitted three outliers gave the same result.

In 15 cases, significant change was evident in COPM scores at 1-year post-DBS despite no change in BFMDRS-M score,

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Table 3 – COPM and BFMDRS results.

	MD type	Classification	COPM performance change		COPM satisfaction change		BFMDRS movement score		
			6 m post DBS	1 y Post DBS	6 m post DBS	1 y Post DBS	Baseline	6 mo	1 year
1	Primary/primary	Idiopathic primary dystonia	5.4[a]	6[a]	6[a]	6.6[a]	24	3 (87%)	5 (79%)
2	plus syndrome	Idiopathic primary dystonia	2.9[a]	6.2[a]	2.2[a]	8[a]	59.50	21 (65%)	10 (83%)
3		Idiopathic primary dystonia	6[a]	8.4[a]	5[a]	8.6[a]	57	16 (57%)	23 (60%)
4		Idiopathic primary dystonia	NT	7.8[a]	NT	7.9[a]	50	18 (64%)	6 (88%)
5		Idiopathic primary dystonia	−0.6	3.3[a]	0.48	4.34[a]	38	30 (27%)	16.5 (38%)
6		Idiopathic primary dystonia	1.17	0.6	1.17	1.4	34	39.5 (−16%)	35 (−3%)
7		Primary plus	6.5[a]	7[a]	7.8[a]	6.2[a]	98	85 (13%)	70.5 (28%)
8		Primary plus	−0.1	0.8	0	1.7	60	72 (−20%)	57.5 (4%)
9		Primary plus	NT	0.13	NT	1.24	75	75 (0%)	60.5 (19%)
10	Secondary – CP	Secondary CP	NT	1.87	NT	1.1	109.50	105.5 (4%)	101.5 (7%)
11	Secondary-non CP	Secondary CP	3[a]	2.8[a]	3.5[a]	4.1[a]	111.50	111.5 (0%)	107 (4%)
12		Secondary CP	0.5	2.7[a]	1.9	3.3[a]	66	NT	77.5 (−17%)
13		Secondary CP	NT	0.4	NT	0.6	66.50	63 (5%)	71 (−7%)
14		Secondary CP	NT	−0.2	NT	−0.3	92.50	97 (−11%)	89 (4%)
15		Secondary CP	1.5	3[a]	1.7	4.7[a]	102.5	102.5 (0%)	93 (9%)
16		Secondary CP	1.4	1.8	1.1	1.8	87.50	97 (−11%)	84 (4%)
17		Secondary CP	3.4[a]	3.5[a]	4.9[a]	4[a]	102.50	103.5 (−1%)	93 (9%)
18		Secondary CP	1.66	2.38[a]	2.6[a]	2.8[a]	67.50	60.5 (10%)	59.5 (12%)
19		Secondary CP	NT	1.9	NT	2.9[a]	112.50	81 (28%)	102.5 (9%)
20		Secondary CP	0.8	2.2[a]	1	2[a]	106.50	98.5 (7%)	97 (9%)
21		Secondary CP	2[a]	2[a]	2[a]	1.3	100	98.5 (2%)	98.5 (2%)
22		Secondary CP	5.5[a]	7.3[a]	5.4[a]	7.4[a]	98	101 (−3%)	106.5 (9%)
23		Secondary CP	2.9[a]	1.5	2.3[a]	0.9	97	93 (4%)	101 (−4%)
24	Secondary	Secondary non CP (metabolic)	1.4	3.7[a]	4.7[a]	6.8[a]	110	108 (2%)	104 (5%)
25	dystonia – non CP	Secondary non CP (metabolic)	NT	−0.1	NT	−0.2	105	105.5 (0%)	108 (−3%)
26		Secondary non CP (stroke)	−0.8	0.7	−0.4	2[a]	27.50	27 (2%)	29 (−5%)
27		Secondary non CP (PKAN)	NT	3.3[a]	NT	3.4[a]	87	98.5 (−13%)	97.5 (12%)
28		Secondary non CP (NBIA)	1.7	2.6[a]	1.3	2.4[a]	64.50	57.5 (11%)	62 (4%)
29		Secondary non CP (Neuromsucular)	3[a]	3.3[a]	4.3[a]	4.8[a]	52.50	74.5 (−42%)	64 (−22%)
30		Secondary non CP (unknown)	2.6[a]	2.80[a]	2.4[a]	2.80[a]	88.50	88.5 (0%)	96.5 (9%)

MD = movement disorder, CP = cerebral palsy, PKAN = pantotheneate Kinase = associated neurodeneration, NBIA = neurodegeneration with brain iron accumulation.
[a] Clinical significant change.

while only one case with primary dystonia showed improvement at 6 months post-DBS in BFMDRS-M score that did not reflect a change in functional performance as measured by COPM scores.

Seven cases failed to show significant change in either COPM or BFMDRS scores. However, when cases were individually reviewed, all but 2 had demonstrated significant change in at least some of the goals identified preoperatively.

The majority of children with primary dystonia achieved improvements in both impairment (BFMDRS) and functional (COPM) outcome measures at 1 year. Only one of three primary plus syndromes achieved either COPM or BFMDRS improvements at 1 year, with those cases presenting with Parkinsonian features failing to demonstrate meaningful improvement in either scale.

Of the secondary dystonia cohort, clinically significant improvements in COPM scores were seen in 11 children at 6

Table 4 – BFMDRS & COPM median and quartiles.

Classification	6 months			1 year		
	Median (centiles 25th–75th)			Median (centiles 25th–75th)		
	BFMDRS	COPM P	COPM S	BFMDRS	COPM P	COPM S
Primary/Primary Plus	21.05 (−8.09–68.32)	5.3 (4.3–8.1)	5.7 (3.8–8.6)	56.58 (11.75–81.18)	8.6 (3.8–9.2)	9.2 (4.0–9.6)
Secondary CP	1.82 (−0.73 to 6.39)	5 (4.4 to 5.2)	5.2 (4.0 to 6.2)	4 (−3.49 to 8.90)	5.6 (5.0 to 6.7)	5.8 (4.8 to 7.2)
Secondary non CP	−13.22 (−27.56 to −6.61)	5.3 (3.9 to 6.1)	5.1 (3.7 to 6.3)	−12.07 (−16.97 to −10.55)	5.7 (4.4 to 6.9)	6.3 (4.0 to 7.3)

At both 6 and 12 months a greater improvement in BFMDRS score was seen in the primary compared to secondary sub-groups (median 25th to 75th centile) at 6 months in primary/primary-plus group 21.05 (−8.09 to 68.32), secondary-CP 1.82 (−0.73 to 6.39) and secondary-non-CP -13.22 (−27.56 to −6.61).

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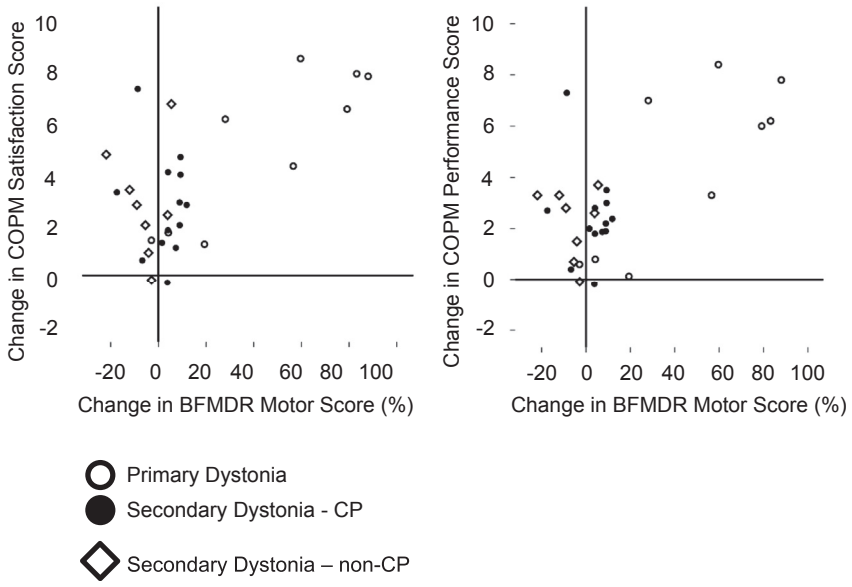

Fig. 2 – Correlation between COPM and BFMDRS scores.

months and 15 children by 1 year, yet none of those showing improvement in COPM scores had significant change in BFMDRS scores at either 6 months or 1 year.

4. Discussion

This small open-label case series study has explored the effect of DBS in paediatric dystonic movement disorders by measuring success in functional goal-achievement after 1 year of neuromodulation. Our findings demonstrate that children with dystonia can benefit from neuromodulation independently of dystonic phenotype and that DBS can meaningfully alter perception of performance and satisfaction relating to specific functional concerns in both primary and secondary dystonia. A correlation between changes in BFMDRS-M scores and COPM-P scores was found only in primary dystonias, indicating a dissociation between the BFMDRS and goal achievement in secondary dystonias. Functional gains were possible without changes detected by BFMDRS and BFMDRS changes were also not necessarily translated into COPM changes.

Secondary dystonias are a heterogeneous group in terms of age at onset, developmental milestones, duration of dystonia at age of DBS and function, presenting assessment challenges.[8,13,18] Use of a single assessment tool applied across a wide spectrum of functional impairments and aetiology is challenging and may fail to capture meaningful responses to management.[13]

Some authors have recommended the use of gross motor function measures to evaluate functional recovery following DBS for children with CP.[15,34] As with other measures of motor ability, e.g. upper limb function measures, such tools are typically restricted to children with a certain level of functional ability.[36] Koy et al., 2013 have performed a meta-analysis of 68 children with secondary-static/CP dystonias indicating a mean improvement in BFMDSRS of about 20%.[19] The COPM, unlike most other outcome measures, has the potential to be used as a common measure across diverse patient groups, potentially allowing, in conjunction with other outcome measures and biomarkers, pooling of results across multiple centres to evaluate more confidently the response to treatment. Such pooling could lead to a better understanding of which patient groups respond best to DBS in relation to motor severity, age, proportion of life lived with dystonia, developmental milestones, aetiological background and in which domains of function.

Limitations to our study include small sample size presenting a risk of type II error, and the lack of blinding and control cases. There is potential for bias in goal-setting and COPM score allocation. We attempted to minimise this by explaining assessment findings prior to the formal goal-setting episode, then endeavouring not to "guide" parents. Further, in order to reduce recall bias, COPM scores from previous time points were not made available to families at follow-up in order to obtain a score related to their current perception of performance and satisfaction for each area of concern. It could be argued that parents have a vested interest in positively reporting outcomes to justify their decision to put their child through an invasive neurosurgical procedure and its associated surgical risks or so as not to disappoint their health-care team.

The importance of establishing realistic expectations when negotiating goals with children and families cannot be underestimated. This can be a challenging prospect for those tasked with formalised goal-setting, typically allied-health professionals, given the paucity of information in the literature regarding functional improvement following DBS in children. Challenges remain in adopting true client-centred goal-setting, with significant knowledge and skill required of therapists in implementing tools such as the COPM and in negotiating realistic goals for intervention. The process of counselling and negotiating goals for DBS needs further exploration, particularly given our limited, but evolving, understanding of what can be achieved through DBS in children. It is important to recognise that a clear differentiation needs to be made between family and health professionals' goals and perceptions: 'small

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improvements' perceived as 'insignificant' by one party, may be viewed as 'significant and worthwhile' by another. Likewise, baseline functional capacity of one child may be the end-point goal for another.

A change in approach is needed to comprehensively evaluate the efficacy of relatively new interventions such as DBS. While it is clear more rigorous studies, including randomised controlled trials, are needed, there are problematic ethical and practical issues in proposing prolonged placebo sham-controlled DBS studies, particularly in paediatrics. Currently, clinicians must rely on their clinical judgement for individual patient selection, with careful goal-setting and judicious evaluation of intervention outcomes. Although prevalent in other paediatric healthcare fields, the use of client-centred measures and evaluation of outcomes related to activity and participation domains of the ICF is largely lacking in paediatric DBS literature. It is important to address such omissions, as only by directly measuring areas of function and adopting a holistic approach to assessment, as advocated by the ICF, will we increase our knowledge of functional recovery and skill acquisition following DBS and evaluate response in terms that are meaningful to children, their families and health-care providers.

5. Conclusion

DBS may produce an improvement in functional performance and satisfaction, as measured by the COPM, in both primary and secondary childhood dystonias. The magnitude of perceived change in individualized COPM goal areas was equivalent regardless of aetiological classification or severity classification. Significant improvements in the secondary dystonia group were seen in the absence of significant changes in the BFMDRS-M score, highlighting the relative insensitivity of purely impairment scales when measuring functional outcomes in this patient group.

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