MCDS Therapy trial – summary of results

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Figure 1: CONSORT flow diagram

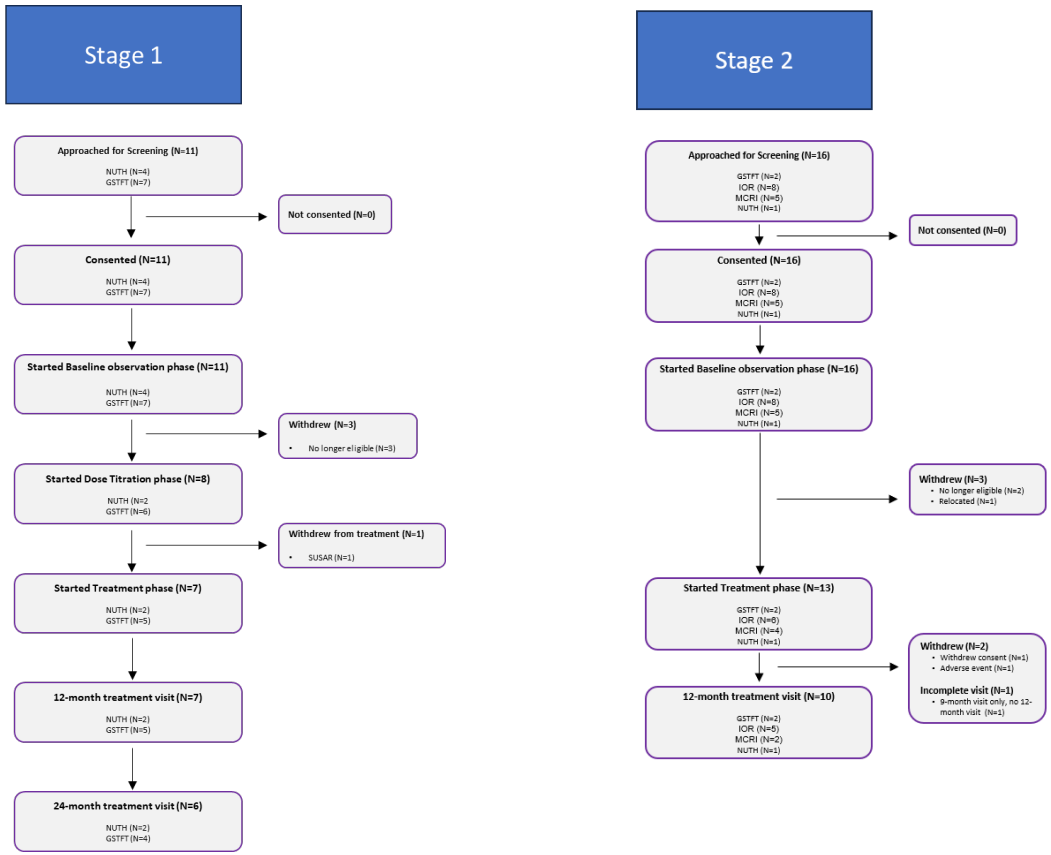

Table 1a: Baseline characteristics (categorical)

Characteristic		N	%
Sex	**Male**	14	66.7
	Female	7	33.3
Pain at diagnosis of MCDS	**Yes**	6	28.6
	No	15	71.4
Surgical interventions to manage MCDS symptoms	**Yes**	7	33.3
	No	14	66.7

Table 1b: Baseline characteristics (continuous)

Characteristic	Number	Mean	Median	SD	Min	Max	Range
Age at baseline visit (years)	21	6.76	6.68	3.26	1.4	12.4	11.0
Height, or length if age <2 years (cm)	21	102.6	108.0	16.0	73.0	128.3	55.3
Height, or length if age <2 years (zscore)[#]	21	-3.16	-3.36	1.12	-5.00	-0.60	4.40
Sitting height, age ≥ 5 years (cm)	17*	64.2	65.1	6.1	51.1	75.3	24.2
Sitting height, age ≥ 5 years (zscore)	17*	-1.09	-1.09	1.14	-3.02	1.47	4.49
Crown-rump length, age < 5 years (cm)	5[£]	54.4	55.5	2.72	49.8	56.8	7.0
Age at onset of first symptoms (years)	21	1.23	0.83	1.16	0.0	4.0	4.0

Table 2: Primary outcome – change in height between baseline and treatment periods

Model parameter	Coefficient	Standard Error	P-value	95% Confidence Interval	
				Lower	Upper
Constant	95.63	1.38	<0.001	92.92	98.33
Growth rate during baseline observation period (cm/week)	0.0854	0.0085	<0.001	0.0687	0.1022
Change in growth rate between baseline and treatment periods (cm/week)	**0.0138**	**0.0094**	**0.144**	**-0.0047**	**0.0323**

Age was centred on 5 years and so the ***constant*** term in the model is interpreted as the estimated mean

Table 3a: Number and percentage of participants (N=21) affected by each type of adverse event, reported by System Organ Class and CTCAE grade

Event term	Grade 1	Grade 2	Grade 3	Total	
				N	%
Infections and infestations	16	0	0	16	76.2
Gastrointestinal disorders	13	0	0	13	61.9
General disorders and administration site conditions	13	0	0	13	61.9
Nervous system disorders	12	0	0	12	57.1
Musculoskeletal and connective tissue disorders	8	2	0	10	47.6
Respiratory, thoracic, and mediastinal disorders	8	0	0	8	38.1
Skin and subcutaneous tissue disorders	8	0	0	8	38.1
Investigations	6	0	0	6	28.6
Eye disorders	5	0	0	5	23.8
Immune system disorders	5	0	0	5	23.8
Ear and labyrinth disorders	3	0	1	4	19.0
Injury, poisoning and procedural complications	4	0	0	4	19.0
Surgical and medical procedures	0	2	0	3	14.3
Metabolism and nutrition disorders	2	0	0	2	9.5
Psychiatric disorders	2	0	0	2	9.5
Blood and lymphatic system disorders	1	0	0	1	4.8

Table 3b: Number and percentage of participants (N=21) affected by each type of adverse reaction, reported by System Organ Class and CTCAE grade

Event term	Grade 1	Grade 2	Total	
			N	%
Gastrointestinal disorders	9	0	9	42.9
Nervous system disorders	9	0	9	42.9
General disorders and administration site conditions	7	0	7	33.3
Skin and subcutaneous tissue disorders	5	0	5	23.8
Eye disorders	4	0	4	19.0
Investigations	3	0	3	14.3
Musculoskeletal and connective tissue disorders	2	1	3	14.3
Infections and infestations	1	0	1	4.8
Psychiatric disorders	1	0	1	4.8
Respiratory, thoracic, and mediastinal disorders	1	0	1	4.8

Table 3c: Chronological listing of serious adverse events (SAEs).

Patient ID	Description	MedDRA Preferred Term	CTCAE Grade	Relationship to treatment	Onset Date	Date of resolution	Duration (days)	Action taken in relation to SAE
11001	Genu valgum	Knee deformity	Grade 2	Possible	08/04/2021	05/08/2022	485	Treatment discontinued Non-drug therapy given
11003	Epiphysiodesis 8-plate insertion	Epiphysiodesis	Grade 2	Unrelated	01/08/2021	01/08/2021	1	Treatment adjusted/interrupted Concomitant Medication Hospitalisation
11003	Epiphysiodesis 8-plate removal	Epiphysiodesis	Grade 2	Unrelated	28/02/2023	28/02/2023	1	Concomitant Medication Hospitalisation
11202	Epiphysiodesis 8 plate removal both hips	Epiphysiodesis	Grade 2	Unrelated	07/12/2023	08/12/2023	2	Treatment adjusted/interrupted Hospitalisation
15205	Acute mastoiditis with removal of adenoids and insertion of bilateral grommets	Mastoiditis	Grade 3	Unrelated	08/08/2023	21/08/2023	14	Treatment adjusted/interrupted Concomitant Medication Non-drug therapy given Hospitalisation