Supplemental Table 1. Descriptive statistics of pediatric-onset patients with DM1 (n = 12)

Variables	n	Time 1 Median (IQR)	Time 2 Median (IQR)	Difference Median (95% CI)	Time 1 Mean (SD)	Time 2 Mean (SD)	Difference Mean (SD)	SMD
Sex (Female/male), n		12 (6/6)						
Age at Time 1		41.5 (31.8, 43.5)			37.8 (11.9)			
Education years		12 (12, 13.3)			12.7 (1.9)			
Onset age		15 (14, 16)			14.6 (2.2)			
Disease duration at Time 1		26.5 (17.5, 30.8)			23.3 (12.1)			
CTG repeat length		600 (342.5, 912.5)			772.1 (590.6)			
MMSE	12	28 (26, 30)	28.5 (25.8, 30)	0 (-1.3, 1.3)	27.5 (2.8)	27.6 (2.5)	0.08 (2.4)	0.03
TMT-A	11	101 (95, 115.5)	101 (86, 137)	-6 (-18.5, 24.9)	108.6 (32.0)	110.6 (37.5)	2 (39.0)	0.05
TMT-B	11	101 (95.5, 149)	117 (95, 143.5)	-4 (-11, 17)	123.8 (49.6)	125.8 (43.6)	2 (25.0)	0.08
Block design	11	33 (27.5, 34)	33 (23.5, 38)	-4 (-4.4, 1.3)	31.8 (9.5)	30.2 (10.5)	-1.64 (5.0)	-0.33
Block design (scaled)	11	4 (3, 5.5)	5 (3.5, 7)	1 (-0.4, 1.5)	4.8 (2.7)	5.5 (2.7)	0.64 (1.7)	0.37
Digit span: forward	10	6 (5, 6)	5.5 (5, 7)	0 (-0.5, 1.2)	5.8 (0.8)	6.1 (1.3)	0.3 (1.4)	0.21
Digit span: backward	10	4 (4, 5)	5 (4, 5.8)	0.5 (0, 1)	4.4 (0.8)	4.9 (0.9)	0.5 (0.9)	0.59
SDMT	10	47.5 (45.5, 52.3)	43.5 (40.3, 49)	-3.5 (-6.4, -1.2)	46.6 (11.0)	42.9 (11.5)	-3.7 (4.4)	-0.84
WCST CA	9	5 (2, 5)	6 (4, 6)	1 (-0.1, 2.4)	3.9 (1.9)	5.0 (1.4)	1.1 (2.2)	0.52
WCST TE	9	15 (12, 29)	11 (10, 15)	-4 (-11.3, -1.7)	19.3 (8.4)	13.1 (5.5)	-6.2 (8.1)	-0.77
MFI: fatigue	10	70.5 (62, 75)	73.5 (67.25, 75)	4.5 (-2.7, 6.5)	68.6 (9.3)	70.7 (8.1)	2.1 (7.7)	0.27
ESS: daytime sleepiness	12	8 (7, 10.3)	10.5 (6, 13)	1 (-0.9, 3.8)	9.3 (5.3)	10.5 (4.7)	1.3 (4.4)	0.28
Apathy	12	18.5 (17, 20.8)	19.5 (12.75, 20)	0 (-4.4, 1.1)	18.8 (3.8)	17.3 (5.3)	-1.6 (5.1)	-0.31
INQoL: QoL score	9	51.7 (49.8, 63.1)	65 (53.9, 69.7)	13.3 (-4.1, 25)	52.7 (14.9)	63.1 (14.7)	10.4 (21.5)	0.48

Median with interquartile range and mean with SD were both reported for description of the sample.
DM1: myotonic dystrophy type 1; IQR: Interquartile range (Q1, Q3); CI: confidence interval; SMD: standardized mean difference; MMSE: Mini-mental State Examination; TMT: Trail Making Test; SDMT: Symbol digit modalities test; WCST: Wisconsin Card Sorting Test; CA: categories achieved; TE: total errors; MFI: Multidimensional Fatigue Inventory; ESS: Epworth Sleepiness scale; INQoL: Individualized Quality of Life.

Supplemental Table 2. Descriptive statistics of adult-onset patients with DM1 (n = 20)

Variables	n	Time 1 Median (IQR)	Time 2 Median (IQR)	Difference Median (95% CI)	Time 1 Mean (SD)	Time 2 Mean (SD)	Difference Mean (SD)	SMD
Sex (Female/male), n		20 (9/11)	-	-				
Age at Time 1		43 (37.5, 50.3)			43.3 (11.7)			
Education years		12 (12, 12.3)			12.45 (1.5)			
Onset age		30 (23.8, 34.3)			29.2 (7.1)			
Disease duration at Time 1		13.5 (4.8, 23)			14.1 (9.8)			
CTG repeat length		825 (581.3, 1193.8)			873.6 (473.2)			
MMSE	20	28.5 (25.8, 30)	26.5 (24, 30)	0 (-2.6, 0.1)	27.4 (3.1)	26.2 (3.9)	-1.2 (3.2)	-0.38
TMT-A	19	118 (89, 145)	128.0 (90, 177)	19 (5.2, 73.1)	143.3 (90.0)	179.1 (141.1)	35.8 (78.1)	0.46
TMT-B	16	130.5 (112.8, 172.8)	154.5 (112, 193.8)	26 (-3.7, 62.1)	149.4 (66.4)	176.0 (87.7)	26.6 (70.2)	0.38
Block design	18	21.5 (20, 33)	24 (13, 29.8)	-2.5 (-4.8, -0.2)	26.5 (12.0)	23.9 (11.9)	-2.6 (5.2)	-0.5
Block design (scaled)	18	3.5 (3, 5.8)	4 (2.3, 5.8)	0 (-0.7, 0.6)	4.4 (2.6)	4.4 (2.6)	-0.1 (1.4)	-0.04
Digit span: forward	18	6 (5, 7)	5 (5, 6)	0 (-1, 0)	6.0 (1.4)	5.5 (1.1)	-0.5 (1.2)	-0.43
Digit span: backward	18	4 (4, 5)	4 (4, 5)	0 (-0.7, 0.7)	4.3 (1.3)	4.3 (1.0)	0 (1.5)	0
SDMT	17	42 (35, 46)	38 (25, 43)	-4 (-8.2, -2.1)	38.8 (9.3)	33.7 (12.8)	-5.1 (6.6)	-0.76
WCST CA	18	4 (2, 5)	4 (1, 5)	-1 (-1.5, 0.8)	3.5 (1.8)	3.1 (2.4)	-0.4 (2.6)	-0.15
WCST TE	18	15 (13.3, 22.8)	18 (14, 24.8)	2.5 (-1.9, 5.8)	18.9 (9.3)	20.7 (10.1)	1.8 (8.6)	0.21
MFI: fatigue	20	62 (52.3, 70.3)	62 (57, 68.5)	1.5 (-3, 6.9)	61.7 (12.6)	63.5 (10.1)	1.9 (11.4)	0.16
ESS: daytime sleepiness	20	7.5 (4, 10)	10 (6, 11.3)	1.5 (-0.1, 3.4)	7.2 (4.2)	8.9 (4.1)	1.7 (4.0)	0.42
Apathy	20	20 (16.8, 22)	20.5 (17, 24)	1 (-0.4, 3.3)	18.3 (6.3)	19.8 (6.0)	1.5 (4.3)	0.35
INQoL: QoL score	12	50 (34.7, 71.3)	56.15 (40.3, 75.4)	5.9 (-0.3, 12.4)	51.5 (26.8)	57.1 (24.5)	5.6 (11.7)	0.48

Median with interquartile range and mean with SD were both reported for description of the sample.
DM1: myotonic dystrophy type 1; IQR: Interquartile range (Q1, Q3); CI: confidence interval; SMD: standardized mean difference; MMSE: Mini-mental State Examination; TMT: Trail Making Test; SDMT: Symbol digit modalities test; WCST: Wisconsin Card Sorting Test; CA: categories achieved; TE: total errors; MFI: Multidimensional Fatigue Inventory; ESS: Epworth Sleepiness scale; INQoL: Individualized Quality of Life.

Supplemental Table 3. Group comparisons (pediatric and adult onset groups) of changes between Times 1 and 2 in neuropsychological measures and psychological variables

Measures	Pediatric	Adult	Group comparison	
	Difference	Difference	Wilcoxon rank sum test	
	Median (95% CI)	Median (95% CI)	Z	P-value
ΔMMSE	0 (-1.3, 1.3)	0 (-2.6, 0.1)	-1.16	0.253
ΔTMT-A	-6 (-18.5, 24.9)	19 (5.2, 73.1)	1.29	0.204
ΔTMT-B	-4 (-11, 17)	26 (-3.7, 62.1)	1.14	0.272
ΔBlock design	-4 (-4.4, 1.3)	-2.5 (-4.8, -0.2)	-0.41	0.698
ΔBlock design (scaled)	1 (-0.4, 1.5)	0 (-0.7, 0.6)	-1.53	0.133
ΔDigit span: forward	0 (-0.5, 1.2)	0 (-1, 0)	-1.14	0.264
ΔDigit span: backward	0.5 (0, 1)	0 (-0.7, 0.7)	-0.82	0.432
ΔSDMT	-3.5 (-6.4, -1.2)	-4 (-8.2, -2.1)	-0.61	0.560
ΔWCST CA	1 (-0.1, 2.4)	-1 (-1.5, 0.8)	-1.74	0.084
ΔWCST TE	-4 (-11.3, -1.7)	2.5 (-1.9, 5.8)	**2.01**	**0.044**
ΔMFI: fatigue	4.5 (-2.7, 6.5)	1.5 (-3, 6.9)	-0.20	0.854
ΔESS: daytime sleepiness	1 (-0.9, 3.8)	1.5 (-0.1, 3.4)	0.68	0.506
ΔApathy	0 (-4.4, 1.1)	1 (-0.4, 3.3)	1.45	0.153
ΔINQoL: QoL score	13.3 (-4.1, 25)	5.9 (-0.3, 12.4)	-0.47	0.665

Bold font shows significance in statistical tests (P < 0.05).
CI: confidence interval; MMSE: Mini-mental State Examination; TMT: Trail Making Test; SDMT: Symbol digit modalities test; WCST: Wisconsin Card Sorting Test; CA: categories achieved; TE: total errors; MFI: Multidimensional Fatigue Inventory; ESS: Epworth Sleepiness scale; INQoL: Individualized Quality of Life.
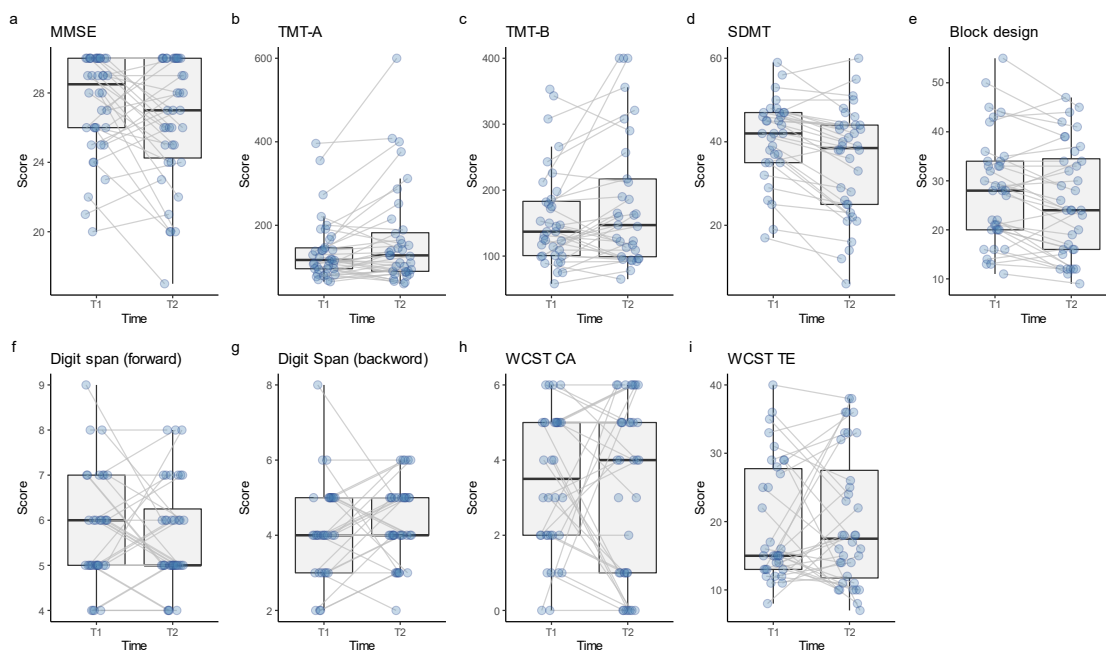
Supplemental Table 4. Logistic regression analysis with death or severe deterioration as outcomes (n = 66)

Neuropsychological performance at Time 1	OR	(95% CI)	SE	P-value
MMSE	0.53	(0.26, 1.00)	0.34	0.061
TMT-A	2.92	(1.42, 7.62)	0.42	**0.011**
TMT-B	3.22	(1.37, 10.80)	0.54	**0.030**
Block design	0.54	(0.22, 1.14)	0.41	0.123
Digit span: forward	0.65	(0.31, 1.24)	0.34	0.202
Digit span: backward	1.38	(0.73, 2.76)	0.33	0.333
SDMT	0.46	(0.17, 1.07)	0.46	0.089
WCST CA	0.38	(0.16, 0.82)	0.42	**0.022**
WCST TE	1.70	(0.86, 3.60)	0.36	0.137

The OR was adjusted for age and sex and was calculated in each model. The explanatory variables included in the logistic regression models were limited to three factors based on the number of events (n = 20) and participants in this study. We entered an explanatory variable of interest (i.e., performance on cognitive tests) and two covariates (age and sex) to estimate the risk of death or severe functional decline.
Bold font shows significance in statistical tests (P < 0.05).
OR: odds ratio; CI: confidence interval; SE: standard error; MMSE: Mini-mental State Examination; TMT: Trail Making Test; SDMT: Symbol digit modalities test; WCST: Wisconsin Card Sorting Test; CA: categories achieved; TE: total errors.
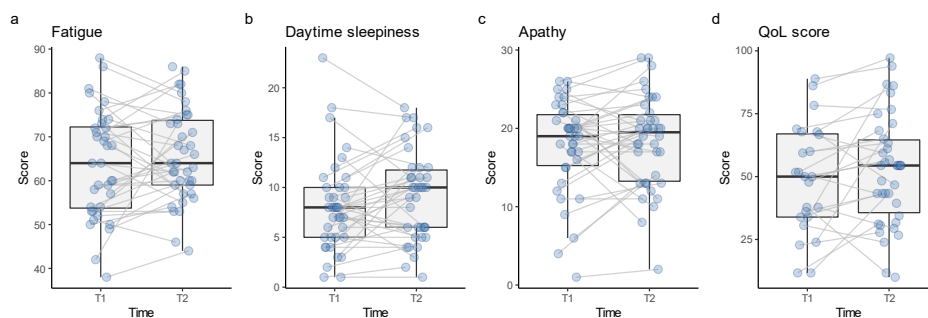
Supplemental Figure 1. Changes over Time 1 and Time 2 in neuropsychological measures (raw scores).

The MMSE, TMT-A, SDMT, and Block design tests showed significant declines in the Wilcoxon signed-rank test ($p < 0.05$).
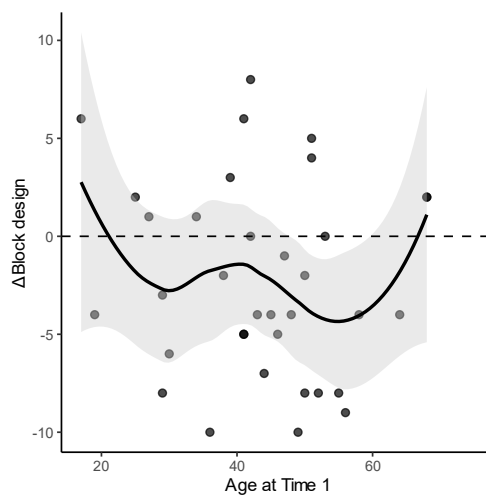
MMSE: Mini-mental State Examination; TMT: Trail Making Test; SDMT: Symbol digit modalities test;

WCST: Wisconsin Card Sorting Test; CA: categories achieved; TE: total errors.

Supplemental Figure 2. Changes over Time 1 and Time 2 in psychological measures (raw scores). For fatigue, daytime sleepiness, apathy, and QoL, higher scores indicated worse symptoms or QoL. The differences were not significant according to the Wilcoxon signed-rank test. The 95% CI of the QoL score was between 0.8–15.0.

QoL: Quality of Life.

Supplemental Figure 3. Locally weighted scatterplot smoothing for age at baseline on delta scores of

Block design in patients