Supplementary Table 1 - Comprehensive Table of Patient Reported Outcome Measures used in Adult Spinal Muscular Atrophy (SMA)

Table Acronym Guide

IC – Internal Consistency

TRT – Test-retest Reliability

CV – Concurrent Validity

R – Responsiveness

MCID – Minimal Clinically Important Difference

SEM – Standard Error of Measurement

MDC – Minimally Detectable Change

SRM – Standardized Response Mean

CT: Clinical Trials with use; IC: Internal Consistency; TRT: Test-Retest Validity; CV: Concurrent Validity; SCC: Spearman's Ranked Correlation Coefficient; PCC: Pearson's Correlation Coefficient; a: Cronbach's Alpha; ES: Cohen's Effect Size

D. C. of Donald	Completed By		Availability & Use		Test Characteristics Validity & Reliability (V & R) Responsiveness (R) Rasch Analysis (RA)			More Information	
Patient Reported Outcome Measure (PROM)	Patient (Y/N)	Caregiver/P roxy (Y/N)	Free (Y/N)	Clinical Trials (Y/N)	V & R (-) indicates Not established in SMA/NMD Population	R (-) indicates Not established in SMA/NMD Population	RA	Additional Information	References
Spinal Muscular Atrophy Functional Rating Scale (SMAFRS)	Y	N	Y	Y	TRT PCC: 0.86 CV "Positive Correlations" to MVICT, 6MWT, PCC: 0.7	R 2 year follow up SRM of -0.61 MCID - SEM - MDC	N	Number of items: 10 items Domains of Assessment: Physical Function Other:	[1–6]
Spinal Muscular Atrophy Health Index (SMA-HI)	Y	N	N	Y	IC α: 0.77-0.96 TRT ICC: 0.6-0.96 CV Subjectively described to differentiate between SMA patient groups.	R - MCID - SEM - MDC	N	Number of items: 107 Symptom Questions Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Pain Systemic Issues Other:	[3,6–8]
Goal Attainment Scale	Y	N	Y	N	IC -	R -	N	Number of items: Variable	[6,9–11]

(GAS)					TRT - CV -	MCID - SEM - MDC -		Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Pain Systemic Issues	
						_		Other: Standardized method of scoring patient goals.	
SMA Independence Scale Upper Limb Module (SMAIS-ULM)	Y	Y	Y	Y	IC - TRT - CV -	R - MCID - SEM - MDC	N	Number of items: 29 Domains of Assessment: Physical Function Communication & Speech Other: The initial validation study has been withdrawn.	[6,12,13]
Activity Limitation Measure (ACTIVLIM)	Y	N	Y	N	IC PCC: 0.96 TRT ICC: 0.87 – 0.93 IER ICC: 0.98 CV Grip Strength PCC: 0.3 and 0.36 Gait Speed PCC: 0.53 MMT SCC: 0.17 to 0.5 FIM* SCC: 0.85	R - MCID - SEM ES: 0.25 SRM: 0.57 MDC Detectable changes more sensitive than overall self-reported change Sensitivity Assessed over 21+/-4 months.	Y	Number of items: 22 Domains of Assessment: Physical Function Other: *Studied in NMD	[3,7,14–18]

					Brooke Grade SCC: -0.63 Vignos Grade SCC: -0.83 FSS PCC: -0.338				
Fatigue Severity Scale (FSS)	Y	N	Y	N	IC α: 0.89 TRT 0.98 CV Fatigue VAS SCC: 0.71 HFMSE PCC: -0.186 to - 0.195 6MWT PCC: -0.527 to - 0.683 INQoL SCC: 0.570 SF-36* SCC: -0.629 SF-36 MCS: PCC: -0.526 SF-36 PCS: PCC: -0.394 MFI (General Fatigue) "Correlated well"	R - MCID - SEM - MDC -	N	Number of items: 9 Domains of Assessment: Fatigue Other: * Limb-Girdle Muscular Dystrophy.	[3,6,18–25]

					ACTIVILIM PCC: -0.338				
Egen Klassifikation 2 Scale (EK2)	Y	N	Y	N	IC - TRT PCC: >0.83 IER ICC: 0.98 IAR ICC: 0.98 CV FVC 0.77 MMT	R - MCID - SEM - MDC -	Y	Number of items: 17 Domains of Assessment: Physical Function Fatigue Communication & Speech Other:	[3,7,26–28]
Rosenberg Self- Esteem Scale (RSES)	Y	N	Y	N	0.87 IC α: 0.75 TRT - CV USER-P PCC: 0.01 – 0.34 Basic Psychological Needs Satisfaction & Frustration Scale (BPNSFN) PCC: 0.36 - 0.66 Satisfaction with Life Scale (SWLS) PCC: 0.43	R - MCID - SEM - MDC -	N	Number of items: 10 Domains of Assessment: Physical Function Mental Health & Cognition Other: *Spina Bifida	[7,29–31]

					*CDRS10 PCC: 0.36				
Duke Health Profile (DUKE)	Y	N	Y	N	IC	R - MCID - SEM - MDC -	N	Number of items: 17 Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Pain Systemic Issues Other: *Data from primary care; non- NMD, Parkerson et al. 1990.	[7,32,33]

Quality of Life – Neuromuscular Disorders (QoL-NMD)	Y	N	Y	N	IC α: 0.77-0.85 TRT ICC: 0.81-0.93 ICC: 0.75 – 0.80 CV WHOQOL-BREF SCC: 0.27-0.72 WHOQOL-BREF SCC: 0.32 – 0.72	R "Scalability assessed to be appropriate" (Dany et al. 2015) MCID - SEM - MDC -	Y*	Number of items: 26 Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Systemic Issues Other: Developed for Slowly progressing NMD, validations were conducted in multiple NMD diagnoses including SMA. *Conducted in French.	[7,34–36]
WHOQOL- BREF	Y	Y	Y	N	IC α: 0.66-0.84 TRT PCC: 0.56 – 0.87 (individual items and domains) CV QOL-NMD SCC: 0.27-0.72 QOL-NMD SCC: 0.32 – 0.72	R - MCID - SEM - MDC	N	Number of items: 26 items Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Pain Systemic Issues Other:	[7,25,35–38]
Neuromuscular Disease Impact Profile (NMDIP)	Y	N	Y	N	IC *α: 0.69 – 0.95 TRT ICC: 0.79 – 0.97 CV SF-36 Muscle Fxn correlates well to Physical Fxn and Lower Extr Activity scales of SF-36.	R ES: Mod – Lar MCID - SEM - MDC	N	Number of items: 36 Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Pain Systemic Issues Other:	[39,40]

								*MND Population	
Satisfaction with Life Scale (SWLS)	Y	N	Y	N	IC α: 0.87 TRT CV RSES PCC: 0.43 USER-P PCC: 0.04-0.43 Basic Psychological	R - MCID - SEM - MDC	N	Number of items: 5 Items Domains of Assessment: Mental Health & Cognition Other:	[7,25,29,41]
Short Form-36	Y	N	Y	Y	Needs Satisfaction & Frustration Scale (BPNSFN) PCC: 0.17-0.51	R	N	Number of items: 36 Items	[7,21,22,25,
(SF-36)					Total Range a: 0.74 – 0.94 TRT Total Range ICC: 0.63 – 0.85 CV NMDIP Physical Function and Lower Extremity Activity scales correlate well with Muscle Function domain. NHP Total Range SCC: -0.46 – -0.72 Physical	ES***: SF-36 Physical Function 1.0 SF-36 Pain 0.3 SF-36 Vitality 0.4 SF-36 MH 0.5 SRM***: SF-36 Physical Function 0.9 SF-36 Pain 0.3		Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Pain Other: *Studied in Limb-Girdle Muscular Dystrophy (LGMD) **Muscular Dystrophy ***Rehabilitation Inpatients *****Studied in NMD	32,42–48]

	T	T
SCC: -0.72		
Pain	SF-36 Vitality	
SCC: -0.70	0.4	
Energy		
SCC: -0.61	SF-36 MH	
Emotions	0.6	
SCC: -0.49		
Social	MCID	
SCC: -0.46	-	
HFMSE	SEM	
Physical	-	
Component	MDC	
SCC: 0.309	-	
Mental		
Component		
SCC: -0.168		
HADS		
Physical		
Component		
SCC: -0.394		
500. 0.371		
Mental		
Component		
SCC: -0.462		
500. 0.402		
FIM****		
PCC: -0.1 – 0.49		
(majority of		
domains between		
0-0.25)		
0-0.23)		
MDC (II		
MRC (Upper		
Limb)		
Physical		
Component		

CCC, 0 100
SCC: 0.198
Mental
Component
SCC: -0.318
MRC (Lower
Limb)
Physical
Component
SCC:0.198
SCC.0.176
Mental
Component
SCC: -0.187
FSS*
SCC: -0.629
FSS to Mental
Component
PCC: -0.526
FSS to Physical
Component
PCC: -0.394
1 CC0.394
Depression*
Depression*
SCC: -0.455
Knee Extension
Maximal
Voluntary
Contraction to
SF-36 PF/SF**
SCC: 0.544-0.609
**NEADL
SCC: 0.533 -
0.751
0.731

	 1	1	<u> </u>	
	**GSES			
	SCC: 0.534 -			
	0.600			
	VAS Pain			
	SCC: -0.476 -			
	-0.715			
	**Checklist			
	Individual			
	Strength			
	SCC: -0.525 -			
	-0.884			
	LICED desire			
	USER*** SF-36 Pain			
	SCC: 0.79 - 0.84			
	SF-36 Vitality			
	SCC: 0.61			
	SF-36 MH			
	SCC: 0.58 – 0.71			
	DUKE Health			
	Profile			
	Total			
	SCC: 0.13 - 0.75 General Health			
	SCC:0.56			
	Physical Health			
	SCC: 0.72			
	Mental Health			
	SCC:0.71			
	Social Health			
	SCC: 0.25			
	ψψΨΙΝΙΟ			
	****INQoL Activities			
	subscale to			
	Physical Function			
	2 mg bloar 1 anothon			

EuroQol-5D-5L	Y	N	Y	Y	scc: -0.59 ****INQoL Pain to SF-36 Pain Subsection Scc: -0.78 ****INQoL Relationships to SF-36 Social Functioning Scc: -0.61 ****Emotions to SF36 Mental Health Scc: -0.55	R	N	Number of items: 6	[7,25,49,50]
EuroQoi-3D-3L	1	IN	ı	1	TRT CV	MCID - SEM - MDC		Domains of Assessment: Physical Function Mental Health & Cognition Pain Other:	[7,23,49,30]
Nottingham Health Profile (NHP)	Y	N	Y	N	IC Total Range α: 0.51 – 0.88 TRT Total Range ICC: 0.73 - 0.95 CV SF-36 Total Range SCC: -0.46 – -0.72	R - MCID - SEM - MDC	N	Number of items: 38 Items Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Pain Systemic Issues Other: *Studied in NMD	[7,42,47,51, 52]

					Physical SCC: -0.72 Pain SCC: -0.70 Energy SCC: -0.61 Emotions SCC: -0.49 Social SCC: -0.46 *Relationships to NHP Social Isolation SCC: 0.54				
Sickness Impact Profile 68 (SIP-68)	Y	N	Y	N	IC - TRT - CV -	R - MCID - SEM - MDC	N	Number of items: 68 Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Other:	[7,53,54]
Utrecht Scale for Evaluation of Rehabilitation Participation (USER-P)	Y	N	Y	N	IC Frequency α: -0.49 to 0.52 *α: 0.70 Restrictions α: 0.89 *α: 0.91 Satisfaction α: 0.82 *α: 0.88	*Small to Moderate (Via ES and SRM) MCID - SEM *5.5 – 6.8 MDC *15.2 - 18.9	N	Number of items: 3 modules with a total of 32 items. Domains of Assessment: Physical Function Mental Health & Cognition Communication & Speech Other: *Validated in outpatient rehab patients. Primarily MSK, TBI, Neurological Diagnoses.	[7,29,55–58]

Between Modules	**Evaluated on TBI or NMD
PCC: 0.14-0.35	patients (Van der Zee et al. 2013)
TRT	
<u>Frequency</u>	
**ICC: 0.65	
<u>Restriction</u>	
**ICC: 0.85	
<u>Satisfaction</u>	
**ICC: 0.84	
CV	
<u>Frequency</u>	
HFMSE (SCC)	
0.26-0.32	
SF-36; pain &	
Fatigue (SCC)	
0.06 - 0.28	
HADS-D (SCC) -0.410.44	
-0.410.44 CISS (SCC)	
-0.09 – 0.21	
-0.09 - 0.21	
Restriction	
HFMSE (SCC)	
0.45-0.59	
SF-36; pain &	
Fatigue (SCC)	
-0.01 - 0.04	
HADS-D (SCC)	
-0.230.23	
CISS (SCC)	
-0.05 – -0.14	
<u>Satisfaction</u>	
HFMSE (SCC)	
-0.04	
SF-36; pain &	
Fatigue (SCC)	
0.21 – 0.42	
HADS-D (SCC)	
-0.410.49	

Barthel Index (BI)	Y	Y	Y	N	IC -	R ES***:	N	Number of items: 10 items	[7,45,47,59– 61]
					Domain SCC: -0.73				
					To Satisfaction				
					Scale (P-Scale)				
					*Participation				
					SCC:0.75				
					Domain				
					*IMPACT-SP To Restrictions				
					*IMDACT CD				
					SCC: 0.59				
					Frequency domain				
					(FAI) to				
					Activities Index				
					*Frenchay				
					PCC: 0.04-0.43				
					SWLS				
					PCC: 0.13 – 0.49				
					Scale (BPNSFN)				
					Needs Satisfaction & Frustration				
					Psychological				
					Basic				
					PCC: 0.01-0.34				
					RSES				
					-0.81				
					PCC: -0.64 -				
					Autonomy				
					**Impact on Participation and				
					-0.000.03				
					CISS (SCC)				

					TRT	0.9		Domains of Assessment: Physical	
					-	~~~		Function	
					CV	SRM***:			
					YYGED.	1.1		Other: Lue et al. identified both	
					USER	MCID		ceiling effects for slow changing	
					SCC: 0.84 – 0. 93	-		NMD and floor effects in a DMD population.	
					FVC*	SEM		p op william.	
					PCC: 0.679	-		*Studied in DMD	
					FEV1*			**Studied in NMD	
					PCC: 0.556	MDC		***Inpatient Rehabilitation	
					MIP*	-			
					PCC: 0.562				
					MEP*				
					PCC: 0.442				
					**INQoL				
					Independence				
					sub-scale				
					SCC: 0.67				
Functional	Y	Y	N	N	IC	R	N	Number of items: 18	[7,17,45,46,
Independence					α: 0.73 - 0.98	ES**:			62,63]
Measure					TRT	Ambulation 1.0		Domains of Assessment:	
(FIM)					-	Self-Care: 0.7		Physical Function	
					CV MFM*	Motor: 0.8		Mental Health & Cognition	
								Fatigue	
					SCC: 0.64 - 0.91	Cognition: 0.1		Communication & Speech	
					SF-36*	SRM**:		Other:	
					PCC: -0.1 – 0.49	Ambulation 0.9		*Studied in NMD	
					(majority of	Self-Care: 1.0		**Rehabilitation Inpatients	
					domains between	Motor:		,	
					0-0.25)	1.1			
						Cognition: 0.2			
					*ACTIVILIM	MCID			
					SCC: 0.85	-			
						SEM			
						MDC			

						-			
Work Productivity and Activity Impairment Questionnaire (WPAI-Q)	Y	N	Y	Y	IC - TRT - CV -	R - MCID - SEM - MDC	N	Number of items: 6 Domains of Assessment: Physical Function Other:	[7,64,65]
Connor Davidson Resiliency Scale (CDRS-10)	Y	N	N	N	TRT - CV *^Fatigue -0.37 *^Quality of Life 0.23 *^Depression -0.54 **RSES PCC: 0.36	R - MCID - SEM - MDC	N	Number of items: 10 Domains of Assessment: Mental Health & Cognition Other: *Long-term Disability Population (MD, Post-Polio, SCI, MS). **Spina Bifida ^Measurement of theoretical latent variables with atypical statistical technique.	[31,66–69]
Individualized Neuromuscular Quality of Life Questionnaire (INQoL)	Y	N	Varies	N	TRT *"Good in most subscales" CV **FSS SCC: 0.570 *SF-36 Physical Function to Activities SCC: -0.59	R *ES 0.21 to - 0.24 Improvement ES -0.090.56 Same ES: -0.26 - 0.21 Deterioration ES: -0.04 - 0.40 MCID -	N	Number of items: 45 Questions Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Pain Other: *Studied in NMD **Studied in Limb-Girdle MD	[21,25,47]

					*BI to				
					Independence	SEM			
					SCC: 0.67	-			
					*Functional	MDC			
					Limitations	-			
					Profile				
					SCC: 0.76				
					*Patient				
					Generated Index				
					SCC: -0.57				
					SCC0.57				
					*INQoL Pain to				
					SF-36 Pain				
					Subsection				
					SCC: -0.78				
					*INQoL				
					Relationships to				
					SF-36 Social				
					Functioning				
					SCC: -0.61				
					*Relationships to				
					NHP Social				
					Isolation				
					SCC: 0.54				
					*Emotions to				
					SF36 Mental				
					Health				
					SCC: -0.55				
Self-Care in Motor	Y	N	Y	N	IC	R	N	Number of items: 15	[70]
Neuron Disease					Internal	-			
Index (SCNMD)					Consistency	MCID		Domains of Assessment:	
					*α: 0.70 - 0.84	=			
						SEM		Other:	
					Content Validity	-		*Evaluated in Motor Neuron	
					*Index - 0.9407	MDC		Disease (ALS, SMA, or MND)	
					*Ratio - 0.78 – 1	-			

					(Interpreted as acceptable to good) TRT Maintenance *ICC: 0.98 Monitoring *ICC: 0.93 Management *ICC: 0.95 CV				
General Self Efficacy Scale (GSES)	Y	N	Y	N	IC - TRT - CV **SF-36 SCC: 0.534 - 0.600	R - MCID - SEM - MDC	N	Number of items: 10 Domains of Assessment: Mental Health & Cognition Other: **Studied in Muscular Dystrophy	[44,71]
Checklist Individual Strength (CIS)	Y	N	Y	N	IC - TRT - CV *SF-36 SCC: -0.525 - -0.884	R - MCID - SEM - MDC	N	Number of items: 20 Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Other: *Studied in Muscular Dystrophy; utilized the Fatigue subscale of CIS.	[44,72]
Patient Reported Outcomes Measurement Information System (PROMIS)	Y	Y (Some)	Y	N	Depth of Validation varies.	Depth of Validation varies.	Var ies	Number of items: Variable Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Pain Systemic Issues	[6,64,66,73– 77]

								Other: PROMIS is a collection of a wide variety of measures all produced by the National Institutes of Health and produce scores from a T score metric based on the general US population. 123 Short Forms, 9 Profiles, covering 102 domains. Many short forms have been used in SMA/NMD studies.	
Neuro-QoL	Y	Y	Y	N	Depth of Validation varies.	Depth of Validation varies.	Var ies	Number of items: Varies Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Communication & Speech Pain Systemic Issues Other: Like the PROMIS, the NeuroQOL is a collection of item banks and short forms which includes 31 item banks and corresponding Short Forms. Over 100 publications in neurological populations.	[12,25,76,78 ,79,79]
Multidimensional Fatigue Inventory (MFI)	Y	N	Depend s upon intende d Use	N	IC Mental Fatigue correlates with all other MFI Domains. General Fatigue correlates with all domains except	R - MCID - SEM - MDC	N	Number of items: 20 Domains of Assessment: Physical Function Mental Health & Cognition Fatigue Other:	[23,80]

T	T			T	madu d			*Unable to determine exact	
					reduced				
					motivation.			correlations via SCC from	
					D1 ' 1 C .'			sources.	
					Physical fatigue				
					only correlated				
					with General and				
					Mental Fatigue.				
					TRT				
					-				
					CV*				
					FSS correlated				
					well to General				
					Fatigue.				
					Age correlated to				
					Mental Fatigue,				
					General Fatigue,				
					Reduced Activity				
					domains				
					SCC: 0.502 –				
					0.647				
Health Utility Index	Y	Y	N	N	IC	R	N	Number of items: 15 (self-	[25,64,81,82
- 3	•	*	1,	11	-	-	1	administered) or 40 (interviewer	1
(HUI)								administered)	,
(1101)					TRT	MCID			
					-	-		Domains of Assessment:	
						SEM			
					CV	-		Other:	
					Significantly	MDC		*Studied in DMD	
					associated with	-			
					patient's current				
					health and ratings				
					of patient's				
					mental status				
Nottingham	Y	N	Y	N	IC	R	N	Number of items: 22	[44,83]
Extended Activities					-	-		-	
of Daily Living					TRT	MCID		Domains of Assessment:	
Scale					_	_		Physical Function	
(NEADL)					CV **SF-36	SEM		Other:	

		SCC: 0.533 -	MDC	**Studied in Muscular Dystrophy	
		BCC. 0.555	WIDC	Studied in Museular Dystrophly	
		0.751	-		