

The Development of a Comprehensive Repository of Patient-Reported Outcome Measures (PROMs) for Spinal Muscular Atrophy and Duchenne Muscular Dystrophy

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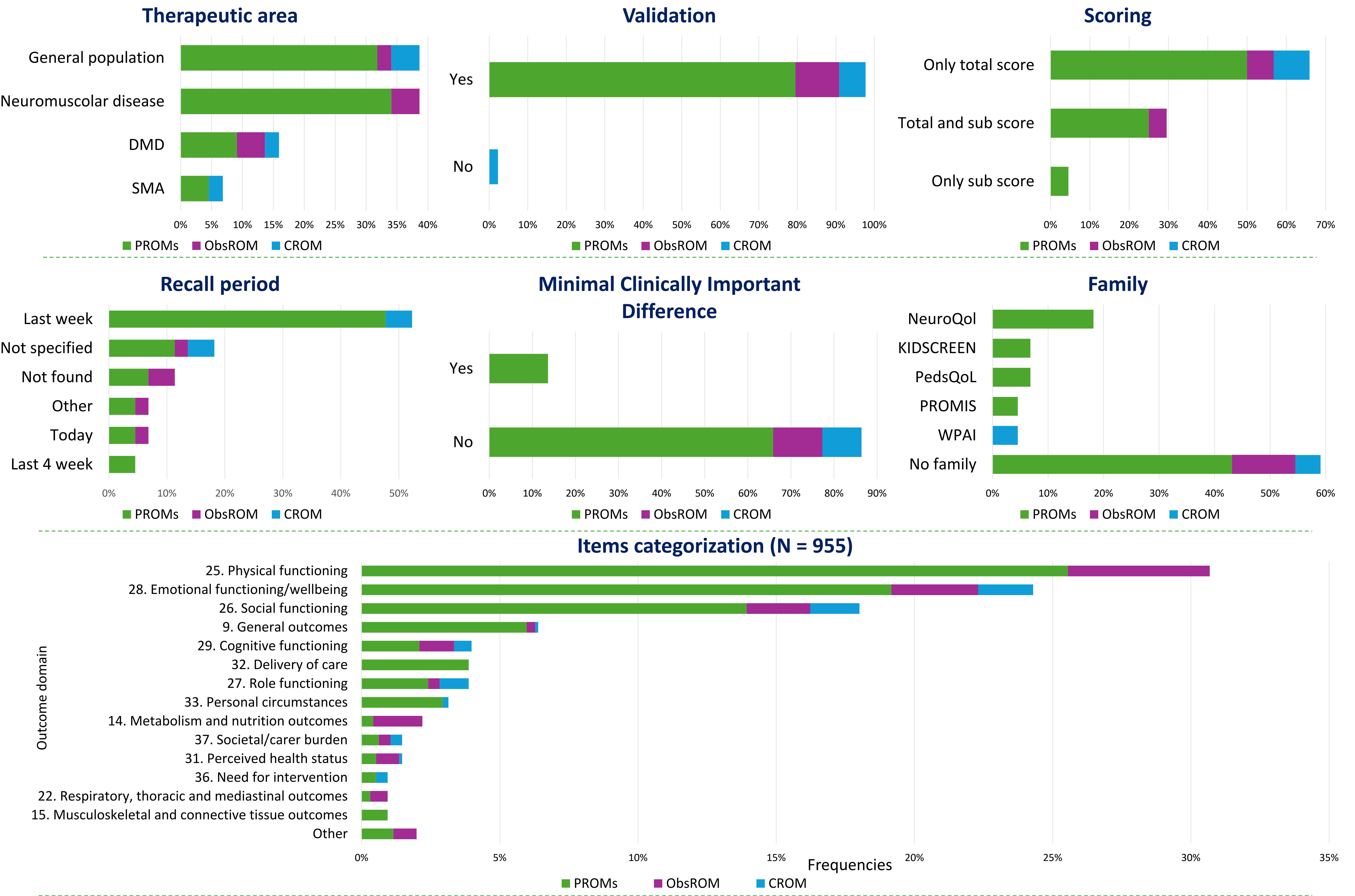
Objective

This study is part of the **PRO4All project**, a multi-stakeholder initiative aimed at raising awareness and knowledge about the current and potential and use of PROs in healthcare. This study aims to create a comprehensive archive of patient-reported outcomes measures (**PROMs**) for Spinal Muscular Atrophy (**SMA**) and Duchenne Muscular Dystrophy (**DMD**), highlighting their key characteristics and target outcome domains. This could improve the selection of appropriate PROMs in clinical settings.

Methods - We aimed to retrieve PROMs, observer-reported measures (ObsROMs) and caregiver-reported outcome measures (CROMs) in SMA and DMD by searching ePROVIDE and published reviews. We developed a data extraction form to collect information on instruments name, type of pathologies, type of questionnaire (i.e., PROM, ObsROM, CROM), recall period (e.g., last week) and number of items. Moreover, we assigned each item a specific domain according to a predefined 38-item taxonomy for outcome classification. We investigated the inclusion of PROMs in publicly available document (clinical study protocols and relative publications, regulatory reports) during the drug development and the approval pathway of SMA/DMD products. (Dodd et al., 2018)

Results

A total of 44 instruments were identified and fully analyzed. The mean number of items per questionnaire was 23 ±17 (range: 5-86).



Integration of PROMs in the Approval Pathway					
Pivotal Study Protocol(s)				EMA	P&R
Product	Pivotal study name	Extraction form protocols	Study publications	EPAR	AIFA innovation report
Spinraza	ENDEAR-SMA1	0	0	0	0
	CHERISH-SMA2/3	4 (GCI-C, PedsQL 3.0 NM, PedsQL 4.0 GS, ACEND)	0	1 (GCI-C)	0
Zolgensma	STR1VE	HIDDEN	0	0	0
Evrysdi	FIREFISH-SMA1	1 (ITQOL-SF47)	1 (ITQOL-SF47)	0	Not innovative
	SUNFISH-SMA2/3	6 (SMAIS, PedsQL NM, PedsQL GS, EQ-5D-5L, WPAI:CG-SMA, Taste questionnaire)	1 (SMAIS)	1 (SMAIS)	
Traslarna	007	2 (PedsQL-GC, PedsQL-MFS, TSQM)	0	1 (PedsQL-GC)	Not innovative
	020	2 (PODCI, ADL)	2 (PODCI, ADL)	1 (PODCI)	
	020e	2 (PODCI, ADL)	0	0	
	019	2 (ADL, Disease Status Survey-DSS)	0	2 (ADL, DSS)	

Conclusions - This study revealed significant heterogeneity in PROMs for SMA and DMD, and a decrease of PROMs consideration throughout the approval pathway. The most frequent outcome domain assigned to individual items was ‘physical functioning’ (30.7 %), followed by ‘emotional functioning/wellbeing’ (24.3 %) and social functioning (18.0 %). The new archive is a valuable tool for guiding researchers and practitioners in choosing suitable measures and promoting a patient-centered approach.