

Understanding physical therapist perspectives on the clinical meaningfulness of 'any point differences' on the Hammersmith Functional Motor Scale-Expanded in SMA

Leslie Nelson,¹ Natalie Land,² Melissa Culhane Maravic,² Thomas Brown,³ Christabella Cherubino,³ Mouhamed Gueye³

¹University of Texas Southwestern Medical Center, Dallas, TX, USA; ²Precision AQ, New York, NY, USA; ³Scholar Rock, Inc., Cambridge, MA, USA

INTRODUCTION

- Spinal muscular atrophy (SMA) is a severe neuromuscular disease characterized by the permanent loss of spinal motor neurons, progressive muscle atrophy, and weakness, which leads to motor function decline¹
- Although diagnosis and treatment of SMA have advanced considerably, patients with SMA report that gaining muscle strength and achieving new motor function skills or functional stabilization remain as unmet needs^{2,3}
- Functional assessments, including the Hammersmith Functional Motor Scale-Expanded (HFMSSE), are important for analyzing treatment efficacy over time⁴; however, determining meaningful changes can be challenging and often require input from patients with SMA or their caregivers
- Understanding which motor function changes are clinically meaningful to patients with SMA, their caregivers, and providers will provide insight into the importance of specific motor function changes and their real-world impact on patients' daily lives

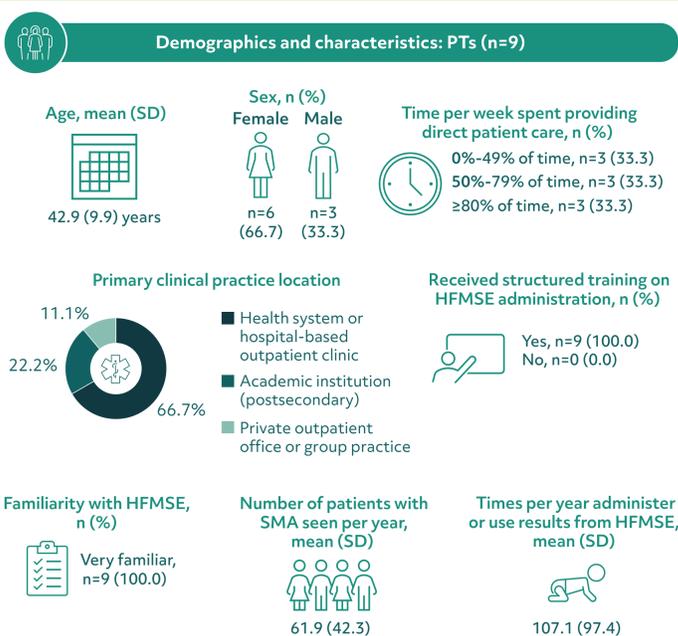
OBJECTIVES

- Examine perspectives on clinically meaningful outcomes among providers (physical therapists [PTs] and neurologists)
- Identify and understand which motor function changes on the HFMSSE are clinically meaningful to providers (PTs and neurologists)
- Inform future research on SMA to incorporate additional clinically meaningful endpoints
- Here, we focus on understanding the PT perspective on the clinical meaningfulness of functional changes measured by the HFMSSE

RESULTS

- Nine of the 20 eligible providers interviewed were PTs (Figure 2)
- PTs' responses per HFMSSE task grouping are listed in Table 2
- For outcomes from neurologist interviews, please see poster 109, also by Nelson and colleagues

Figure 2. PT demographics and characteristics



HFMSSE, Hammersmith Functional Motor Scale-Expanded; PT, physical therapist; SD, standard deviation; SMA, spinal muscular atrophy.

Table 2. Number of PT responses for each HFMSSE task grouping

HFMSSE item number	Task	PTs (n=9)
1	1 Plinth/chair sitting	1
2	2 Long sitting, legs straight	2
3, 4	3 One hand to head in sitting 4 Two hands to head in sitting	4
5-9	5 Supine to side-lying 6, 7 Rolls prone to supine over R/L 8, 9 Rolls supine to prone over R/L	2
10, 14	10 Sitting to lying 14 Lying to sitting	4
11	11 Props on forearms	3
13	13 Props on extended arms	4
12, 17	12 Lifts head from prone 17 Lifts head from supine	3
15, 16	15 Four-point kneeling 16 Crawling	2
18-20	18 Supported standing 19 Unsupported standing 20 Stepping	3
21-27	21, 22 Hip flexion in supine (R/L) 23, 24 High kneeling to half kneel (R/L) 25, 26 High kneeling to stand leading with R/L leg 27 Stand to sit	6
28, 29	28 Squat 29 Jump 12"	4
30-33	30 Ascends stairs with rail 31 Descends stairs with rail 32 Ascends stairs without rail 33 Descends stairs without rail	6

HFMSSE, Hammersmith Functional Motor Scale-Expanded; L, left; PT, physical therapist; R, right.

METHODS

- 60-minute, web-based, in-depth qualitative interviews were conducted with providers (PTs and neurologists) experienced with treating SMA and using the HFMSSE; this analysis focuses on interviews with PTs
- PTs meeting the inclusion criteria were eligible for study enrollment (Table 1)

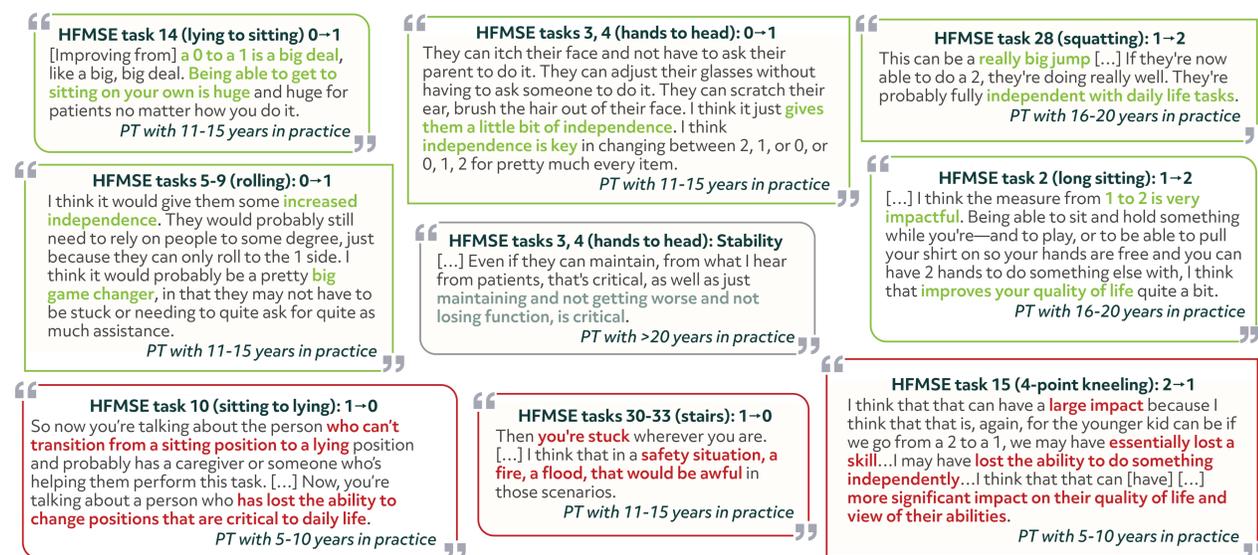
Table 1. Inclusion criteria

Key inclusion criteria	Description
	Licensed PT with a Bachelor, Master, or Doctor of Physical Therapy currently practicing in the United States
	Provides care/services to patients with SMA
	Reports being familiar or very familiar with HFMSSE
	Administers or uses results from the HFMSSE assessment to evaluate their patients with SMA ≥5 times per year
	Practicing physical therapy for ≥5 years

HFMSSE, Hammersmith Functional Motor Scale-Expanded; PT, physical therapist; SMA, spinal muscular atrophy.

- PTs regarded clinically meaningful changes as repeatable functional improvements that are relevant to the patient and improve QoL (Figure 3)
- When defining clinically meaningful change, PTs had slightly more specific criteria than neurologists who were interviewed (please see poster 109, also by Nelson and colleagues)
- Score changes must reflect true functional changes, rather than random variation or temporary factors (eg, fatigue, time of day)
- Objective score changes alone may not fully capture the real-world impact or what is most important to patients
- Many noted that any point difference or stability across specific HFMSSE tasks can be meaningful, as it could enhance patient independence, engagement in meaningful activities, and QoL (Figure 4)
- Tasks reflecting the most clinically meaningful changes vary according to age and functional level

Figure 4. PT perspectives on clinically meaningful changes reflected in HFMSSE tasks



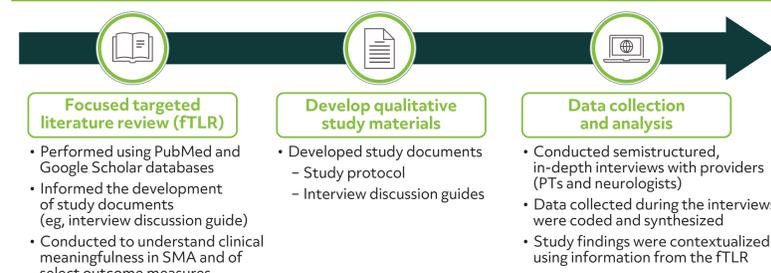
PT quotes have been edited for readability.
HFMSSE, Hammersmith Functional Motor Scale-Expanded; PT, physical therapist.

CONCLUSIONS

- Clinically meaningful change was regarded as repeatable functional changes that are relevant and meaningful to a patient
- PTs generally recognized that any point differences or stability in HFMSSE scores are meaningful, as these reflect important impacts on patients' independence, ADLs, and QoL
 - Stability was generally regarded as important to patients' QoL and independence, as functional declines have meaningful negative impacts
- Understanding the clinical meaningfulness of HFMSSE for individuals is essential to assist in relating point changes to real-world differences in patient experiences

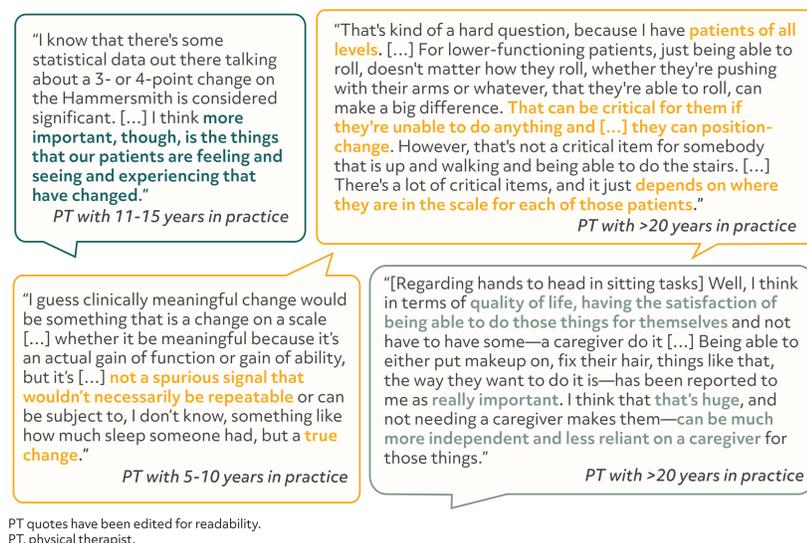
- A semistructured discussion guide was informed by a focused targeted literature review (Figure 1)
- The discussion guide included open-ended questions to elicit and examine perspectives and experiences on:
 - How clinical meaningfulness is interpreted
 - What constitutes meaningful change at the individual item level on the HFMSSE
 - How meaningful change in specific motor function abilities may impact the patient's ability to perform activities of daily living (ADLs), quality of life (QoL), independence, and psychosocial health
- Interview transcripts were analyzed using the constant comparative method, an iterative approach to aggregate and synthesize qualitative data

Figure 1. Study approach overview



FTLR, focused targeted literature review; PT, physical therapist; SMA, spinal muscular atrophy.

Figure 3. PT perspectives on clinical meaningfulness



PT quotes have been edited for readability.
PT, physical therapist.

References

- Mercuri E, et al. *Nat Rev Dis Primers*. 2022;8(1):52.
- Cure SMA. 2023 State of SMA report. Accessed Feb 18, 2026. https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb-1.pdf
- Parsons JA, et al. *Ann Clin Transl Neurol*. 2025;12(10):2020-2035.
- Ramsey D, et al. *PLoS One*. 2017;12(2):e0172346.

Acknowledgments

- This study was funded by Scholar Rock, Inc.
- Medical writing and editorial support were provided by Aidan Moriarty, PhD, and Dena McWain of Helios Global Group, and funded by Scholar Rock, Inc.
- Project management support was provided by Taryn Bosquez-Berger, PhD, CMPP, of Scholar Rock, Inc.
- We thank Rozanne Wilson, PhD, Claire Cagle, MPH, and Amal Jama'eddine of Precision AQ for their important contributions to this project

Disclosures

LN is a consultant for AveXis, Biogen Inc., F. Hoffmann-La Roche, Novartis, and Scholar Rock, Inc. NL is an employee of Precision AQ. MCM is an employee of Precision AQ and owns equity interest in Precision AQ's parent company, Precision Medicine Group. TB, CC, and MG are employees of and stockholders in Scholar Rock, Inc.



To download a copy of this poster, scan QR code.