

Patient Perspective on Clinical Trials and Ratings Scales

Emma C.

Disclosures:

- None

My Background and Experience:

Diagnosis:

- Spinal Muscular Atrophy (SMA) Type III

Clinical Trial Participation:

- 10 years in Biogen Spinraza Trials
 - Aged 3-13
- 1 year in Pierre Thecaflex Device Trial
 - February 2025



Why I Participate in Clinical Trials:

Parents decision to enroll me (2013)

- Opportunity to get access to life-changing drug
- Explained my participation could help others like me

15 years old, my choice was to enroll (2025)

- I hoped the device would help make things easier for me
- I wanted to help my sister

Reasons why clinical trials are challenging for me:

Pressure:

- Afraid of low or “bad” scores meaning I would lose access to the drug
- Afraid my scores would prevent other people from getting the drug

Consequences:

- Anxiety and stress over my performance
- Physical pain and exhaustion

Significance

- Data demonstrates THAT day results and does not consider variability
- Testing things I don't do daily or that do not matter to me

Specific issues with the 6MWT:

- **Pushed beyond limit**
- Mental fortitude / physical strength
- **Torturous**
 - Silent, caused pain, posed risk of injury
 - Testing areas = intimidating
- **Pain scales**
- Irrelevant to my day-to-day life



Specific Issues with the Hammersmith test:

- Confronting my disease and limits head-on
- **Risk of injury**
- Impossible tasks
- **Stress - physical and mental**
- Test variability
- **Clinical verses everyday life**



What I Would Like to See Measured in Trials:

- **Relevant measurements:**
 - how is the participant's daily life impacted/changed
- **Alternative measurements**
 - Wearable
- **Questionnaires**
 - Consider WHEN to ask the questions



Thank you.

I appreciate this opportunity to share my experience.