Design and Validation of a Specific Clinical Outcome Measure for Adolescent and Adult Patients with Spinal Muscular Atrophy: An Interim Rasch Analysis

PCR240

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Supplemental materials

Items included in the "toolkit":

▪ Bulbar function

Does the patient have clinical signs of bulbar disease?
Yes/No
Do not continue if the answer is negative

To what extent can the patient perform the following activities of daily living?

1. Make themselves understood when talking to an acquaintance?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

2. Make himself/herself understood when talking to a stranger?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

3. Make himself/herself understood when speaking on the phone?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

4. Talk for hours?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

5. Speak louder to make himself/herself understood in a noisy room?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

6. Drink liquids without choking or coughing?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

7. Swallow pills?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

8. Does the patient notice excess saliva in the mouth?
Never/occasionally/continually

9. Does the patient need nutritional supplements (nutritional shakes)?
He/she doesn't need them/they are a nutritional supplement/they make up the majority of your diet

▪ Axial function

Does the patient have clinical signs of NIM in the axial region?
Yes/No
Do not continue if the answer is negative

1. Shake the head to say yes or no?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

2. Does the patient need to rest his head on the headrest when sitting in the wheelchair?
Needs support continually/needs support at times/does not need support

3. Does the patient need to lean on the backrest when sitting in the wheelchair?
Needs support continually/needs support at times/does not need support To what extent can the patient perform the following activities of daily living?

4. Keep sitting in the toilet?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

▪ Breathing function

Does the patient have a vital capacity greater than 80?
Yes/No
Do not continue if the answer is positive

1. Does the patient have a feeling of shortness of breath?
At rest/when carrying out activities or efforts/never

2. Can the patient cough effectively (expelling mucus) in daily life?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

3. Does the patient use cough assist?
Daily/occasionally (with respiratory infections)/never

4. Does the patient use ventilatory support (invasive and non-invasive ventilation)?
More than 16 h a day/8–16 h a day (at night and occasionally during the day)/less than 8 h a day

▪ Upper limb function

Does the patient have clinical signs of lower motor neuron involvement in the upper limbs?
Yes/No
Do not continue if the answer is negative

1. Use a touchscreen phone or tablet?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

2. Does the patient use the electric chair joystick?
Unable to do it without help/can do it with difficulty or needs an adapted joystick/can do it without difficulty To what extent can the patient perform the following activities of daily living?

3. Use a computer?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

4. Press a switch on the wall (light, elevator, etc.)?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

5. Brush his/her teeth with any type of toothbrush?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

6. Eat and drink independently?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

7. Use a knife and fork (to cut food)?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

8. Move around his/her house in a non-motorized wheelchair?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

9. Reaching for objects on a high shelf:
Unable to do it without help / can do it with difficulty (includes using alternative methods but not help from others) / can do it without difficulty

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Supplemental materials

Lower limb function

To what extent can the patient perform the following activities of daily living?

1. Stay up?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

2. Roll over in bed?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

3. Walk around his/her house?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

4. Can he/she wash his/her body in the shower?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

5. Walk down the street on a flat surface?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

6. Go up a stretch of staircases?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

7. Get up from the ground?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

8. Run?
Incapable of doing it without help/can do it with difficulty (includes the use of substitute methods, but not help from third parties)/can do it without difficulty

Fatigability

1. How long does it take the patient to complete a meal?
Same as the rest of the people (about 30 min)/up to 15 min more than the rest (about 45 min)/more than 15 min more than the rest (more than 45 min)

2. If the patient has applied more effort than usual, does the fatigue last until the next day?
Often/sometimes/never

3. Are there any activities that the patient has been able to do in the morning and that he/she has not been able to do in the afternoon or at night (has he/she run out of battery throughout the day)?
Often/sometimes/never

4. When the patient performs a repetitive daily task (such as writing or walking), do they notice that, after doing it for a while, they perform it increasingly worse or have to stop?
Frequently / sometimes / never

5. Have they been able to maintain their level of energy and activity throughout the entire day?
Frequently / sometimes / never

Other

1. Does he/she have cramps?
Often/sometimes/never

2. Does his/her functionality worsen with cold or humidity?
A lot/some/nothing