Nusinersen MAA Report – additional PROMs data analysis for the adult SMA cohort

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1. Background

1.1 UK SMA Patient Registry

The UK SMA Patient Registry was established in 2008 and has been co-ordinated by Newcastle University within the John Walton Muscular Dystrophy Research Centre (JWMDRC) since 2014.

The registry collects patient-reported data from individuals living with SMA in the UK and in Ireland, while its overall aims are to disseminate SMA-relevant information to participants, support the SMA community, aid the rapid identification of eligible patients for clinical studies, and act as a source of information – including post-marketing surveillance data – to academics, industry and healthcare professionals.

Participation in the registry is patient-initiated via a secure online portal. Patients consent online and then enter their clinical and genetic data via registry questionnaires. These questionnaires ask specific questions about patients' SMA diagnosis and their condition, including their motor function, requirement of assistance for feeding or breathing, scoliosis, contractures, hospitalisations, other illnesses, medications, and participation in clinical trials, in addition to patient-reported outcomes measures (PROMs).

PROMs were integrated into the patient registry in April 2022. The PROMs data collected through the registry has been linked to Adult SMA REACH data, allowing for the alignment of patient-entered and clinician-entered data, and has been presented in the following report.

2. PROMs

Patient-reported outcome measures (PROMs) data was collected through the UK SMA Patient Registry. The patient registry data cut was completed on 25th March 2025.

The registry collects data using the following PROMs:

- EQ-5D-5L – a self-assessed validated instrument covering five dimensions: mobility, self-care, usual activities, pain/discomfort, and anxiety/depression. Each domain has five levels: no problems, slight problems, moderate problems, severe problems, and extreme problems.

  The EQ-5D-5L also includes the EQ VAS – a visual analogue scale asking patients to rate their health on a scale from 0 (the worst health imaginable) to 100 (the best health imaginable) on the day of questionnaire completion.

- SMA independence scale – upper limb module (SMAIS-ULM) – a 22-item validated scale to assess the degree of assistance required to perform typical daily activities in individuals with Type II and non-ambulatory Type III SMA. The

total score ranges from 0 to 44, where a higher score indicates greater independence and less assistance required.

- Patient global impression of severity (PGI-S) – a simple, validated, single-item, self-reported categorical scale to assess the severity of a clinical condition. There are four responses ranging from: not at all, mild, moderate and severe.

- Patient global impression of improvement (PGI-I) - a simple, validated, single-item, self-reported categorical scale to assess the improvement of a clinical condition. There are seven responses ranging from: very much improved, much improved, a little improved, no change, a little worse, much worse and very much worse.

- Free-text comments

2.1 PROMs analytic population

The cohorts within the presented PROMs data match the primary population used in the main statistical report (see section 2.1 of the main statistical report), based on SMA type and WHO motor function status at Adult SMA REACH baseline.

Due to the small number of Type 1 and Type 4 patients, these two cohorts have been excluded from the PROMs analysis. Analysis thus focuses solely on the following cohorts as described in the SAP:

• Type II and Type III: non-sitter

• Type II and Type III: sitter

• Type II and Type III: walker

2.2 PROMs analysis

For the EQ-5D-5L, EQ VAS, and SMAIS-ULM measures, the following analyses have been presented:

1) Baseline descriptives of patients' PROMs data (see section 2.4).

2) Descriptive statistics of PROMs responses over the course of treatment (see section 2.5), correlated to each patient's clinical appointment.

PROMs data from the PGI-S and PGI-I questionnaires has not been presented in this report.

The free-text comments have been presented, without analysis, as an appendix to the main statistical report (see appendix 2 of the main statistical report).

All analysis of the PROMs data was done using Microsoft Excel.

2.3 Limitations

The patient-reported nature of this data holds the risk of voluntary response bias; for example, patients with more/less severe disease being more willing/able to submit responses. There also may have been recall bias present.

The PROMs data obtained from the registry and displayed in this report represents a smaller proportion of the entire SMA population (and specifically, of those treated with Nusinersen) than the data obtained from the analyses described in the main statistical report.

Results must therefore be interpreted with care, especially due to the limited number of responses.

2.4 Baseline descriptives of PROMs responses

This section provides descriptive analysis of each cohort's PROMs data entry at the start of treatment.

Due to the later implementation of PROMs into the registry (April 2022) compared to many patients' date of initiation of Nusinersen treatment, there is minimal baseline PROMs data as defined by data entry within a -6/+3 months baseline window (as used in section 3.1 of the main statistical report) from the date of Nusinersen treatment initiation.

To increase the volume of data for the baseline descriptives of PROMs data, "baseline" in section 2.4 is therefore instead defined as PROMs data entry within +/- 6 months of treatment initiation. Despite this increased window, the baseline patient-reported data presented is still limited by a small sample size. No type II and III walkers submitted in-window baseline data for the EQ-5D-5L, EQ VAS, or SMAIS-ULM questionnaires.

Only 1 response per questionnaire from each respondent has been used for the baseline data. When there has been duplicate data – i.e. when patients have reported data more than just once within the baseline window – only the PROMs entry that is closest to the date of first dose has been used.

2.4.1 EQ-5D-5L

EQ-5D-5L is a self-assessment tool which has been validated and covers five dimensions: mobility, self-care, usual activities, pain/discomfort, and anxiety/depression.

Each dimension consists of five levels, representing: no problems (1), slight problems (2), moderate problems (3), severe problems (4), and extreme problems (5).

A higher value for each dimension in EQ-5D 5L indicates a higher level of problems experienced by the patient.

Tables P1, P2 and P3 present the frequencies and proportions of EQ-5D-5L responses at baseline according to dimension and level.

Table P1: Type II and III non-sitters. Descriptive statistics of EQ-5D-5L responses at baseline, reported by dimension.

	Mobility	Self-care	Activities	Pain	Anxiety
Mean score (SD)	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Range	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Max	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Min	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Median	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Number of responses/respondents	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

Table P2: Type II and III sitters. Descriptive statistics of EQ-5D-5L responses at baseline, reported by dimension.

	Mobility	Self-care	Activities	Pain	Anxiety
Mean score (SD)	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Range	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Max	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Min	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Median	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Number of responses/respondents	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

Table P3: Type II and III walkers. Descriptive statistics of EQ-5D-5L responses at baseline, reported by dimension.

	Mobility	Self-care	Activities	Pain	Anxiety
Mean score (SD)	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Range	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Max	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Min	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Median	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
Number of responses/respondents	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

Fig. P1: Type II and III non-sitters and sitters. Mean average of EQ-5D-5L responses at baseline, reported by dimension and cohort.

2.4.2 EQ VAS

The EQ VAS records the respondent's overall current health on a vertical visual analogue scale, where the endpoints are labelled 'the best health you can imagine' (100) and 'the worst health you can imagine' (0).

In the analysis, the EQ VAS score is treated as a continuous variable.

Table P4 presents descriptive statistics, including mean and median values, for EQ VAS scores at baseline.

Table P4: Descriptive statistics of EQ VAS responses at baseline, by cohort.

Cohort	[illegible]	[illegible]	[illegible]	[illegible]	[illegible]	[illegible]
Type II and III non-sitters	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

2.4.3 SMA Independence Scale – Upper Limb Module (SMAIS-ULM)

The SMA Independence Scale (Upper Limb Module) is a 22-item validated scale which assesses the degree of assistance required to perform typical daily activities in individuals with Type II and non-ambulatory Type III SMA.

The total score range is 0-44, where a <u>higher score</u> indicates <u>greater independence</u> and less assistance required.

While the total possible score of the SMAIS should be 44, some patients have lower total scores due to incomplete questionnaires. The total scores of incomplete questionnaires have therefore been adjusted using the following formula: [Total score]/[Number of questions completed]*[Total number of questions].

In the analysis, the SMAIS-ULM score is treated as a continuous variable.

Table P5 presents descriptive statistics, including mean and median values, for SMAIS-ULM total scores (adjusted) at baseline.

Table P5: Descriptive statistics of SMAIS-ULM responses at baseline, by cohort.

	Type II and III non-sitters	Type II and III sitters	Type II and III walkers
Number of responses/respondents	[redacted]	[redacted]	[redacted]
Mean total adjusted score (SD)	[redacted]	[redacted]	[redacted]
Max	[redacted]	[redacted]	[redacted]
Min	[redacted]	[redacted]	[redacted]
Range	[redacted]	[redacted]	[redacted]
Median	[redacted]	[redacted]	[redacted]

2.5 Descriptives of PROMs responses over course of treatment

This section presents descriptives of PROMs data entry over the course of treatment, in correlation to each patient's clinical appointment.

For the analysis contained in this section, only PROMs data which was entered within a +/- 8-week window of a patient's baseline (BL) or follow-up (FU) clinical appointment, as entered into the Adult SMA REACH database, has been included. In the following tables, time points (e.g. BL, FU 1) refer to the clinic.visit time-point to which each PROMs entry corresponds. Follow-up visits occur at 6-month intervals (+/- 3 months) from the original baseline visit.

Baseline data is therefore defined as PROMs entered within a +/- 8-week window of the patient's baseline clinical visit (as defined in section 3.1 of the main statistical report). Please note that the definition of baseline in section 2.5 of the PROMs report therefore differs from that used in the 2.4 analysis of PROMs baseline descriptives. The number of included "baseline" responses presented may therefore differ between the section 2.4 and 2.5 analyses.

When there has been duplicate data – i.e. when patients have reported data more than just once per clinic visit – the PROMs entry that is closest to the study visit has been used.

2.5.1 EQ-5D-5L

Analysis of EQ-5D-5L responses was conducted using the Pareto Classification of Health Change (PCHC) method. With this approach, an EQ-5D health state is deemed to be 'better' than another if it is better on at least one dimension and is no worse in any other dimension. An EQ-5D health state is deemed to be 'worse' than another if it is worse in at least one dimension and is no better in any other dimension.

Using that principle to compare a patient's EQ-5D health states between any two time periods, there are only four possibilities:

- Their health state is better
- Their heath state is worse
- Their health state is exactly the same
- The changes in health are "mixed": better on one dimension, but worse on another.

Tables P6, P7 and P8 present the change in each patient's EQ-5D-5L response, compared to their previous response, across the three cohorts.

N/A values refer to a patient's first response, which has no previous response to be compared to.

It is important to note that the changes in score represented in this table do not necessarily refer to submissions of the EQ-5D-5L in consecutive time-frames. For instance, in table P6, 1 patient's response entered at the FU 3 (18 (+/-3) months since baseline) visit represented a better health profile compared to their previously

submitted EQ-5D-5L response, but this previous response may have been entered at baseline, FU 1, or FU 2.

Table P6. Type II and III non-sitters. Changes in each patient's EQ-5D-5L health profile, compared to their previous EQ-5D-5L submission.

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	Better n (%)	Same n (%)	Mixed n (%)	Worse n (%)
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

*This was the first EQ-5D-5L submission by this patient while on treatment, so there is no point of comparison to a previous submission.

Table P7. Type II and III sitters. Changes in each patient's EQ-5D-5L health profile, compared to their previous EQ-5D-5L submission.

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	Better n (%)	Same n (%)	Mixed n (%)	Worse n (%)
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

*This was the first EQ-5D-5L submission by this patient while on treatment, so there is no point of comparison to a previous submission.

Table P8. Type II and III walkers. Changes in each patient's EQ-5D-5L health profile, compared to their previous EQ-5D-5L submission.

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	Better n (%)	Same n (%)	Mixed n (%)	Worse n (%)
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

*This was the first EQ-5D-5L submission by this patient while on treatment, so there is no point of comparison to a previous submission.

Fig. P2. Type II and III non-sitters, sitters, and walkers. Proportion of change in health profile of EQ-5D-5L responses compared to patient's previous EQ-5D-5L response. Excludes each patient's first response (N/A).

[redacted]

2.5.2 EQ VAS

Tables P9, P10 and P11 presents the change in each patient's EQ VAS response compared to their previous response.

The tables show the number of instances of the following: any score increase of 10 points or more compared to the patient's previous response, any score increase at all, any decrease at all, and any decrease of 10 points or more.

The columns indicating score changes of 10 points or more must therefore be read as sub-columns of the '1+ point increase/decrease' columns in the table. N/A values refer to a patient's first response, which has no previous response to be compared to.

Results in this table do not necessarily refer to submissions of the EQ VAS in consecutive time-frames. For instance, in table P9, [redacted] respondents' scores entered at the FU 3 visit represented an increased score compared to their previous response, but this previous score may have been entered at baseline, FU 1, or FU 2.

Table P9. Type II and III non-sitters. Change of each patient's EQ VAS score compared to the score of their previous EQ VAS submission, according to visit window (time since baseline)

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	+ >=10 n (%)	+ >=1 n (%)	Same n (%)	- >= 1 n (%)	- >=10 n (%)
7Ï=¾Ìı¾	[redacted]						
[redacted]							
[redacted]							
[redacted]							
[redacted]							
[redacted]							
[redacted]							
[redacted]							
All							
All (without N/A)							

*This was the first EQ VAS submission by this patient, so there is no point of comparison to a previous submission.

Table P10. Type II and III sitters. Change of each patient's EQ VAS score compared to the score of their previous EQ VAS submission, according to visit window (time since baseline).

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	+ >=10 n (%)	+ >=1 n (%)	Same n (%)	- >= 1 n (%)	- >=10 n (%)
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All (without N/A)	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

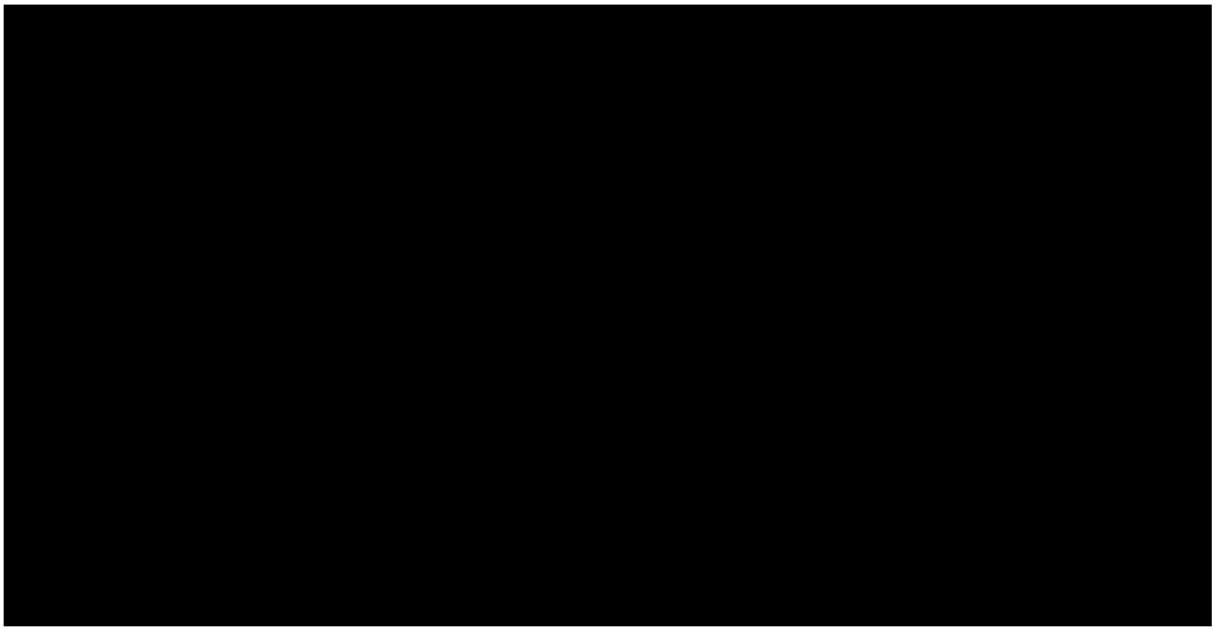
*This was the first EQ VAS submission by this patient, so there is no point of comparison to a previous submission.

Table P11. Type II and III walkers. Change of each patient's EQ VAS score compared to the score of their previous EQ VAS submission, according to visit window (time since baseline).

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	+ >=10 n (%)	+ >=1 n (%)	Same n (%)	- >= 1 n (%)	- >=10 n (%)
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All (without N/A)	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

*This was the first EQ VAS submission by this patient, so there is no point of comparison to a previous submission.

Fig. P3. Type II and III non-sitters, sitters, and walkers. Proportion of change in score of EQ VAS responses compared to patient's previous EQ VAS response. Excludes patient's first response (N/A).



2.5.3 SMAIS-ULM

Tables P12, P13, and P14 present whether each patient's total (adjusted) SMAIS-ULM score increased, decreased, or remained the same compared to the previous SMAIS-ULM questionnaire they submitted.

N/A values refer to a patient's first response, which has no previous response to be compared to.

It is important to that the changes in this table do not necessarily refer to submissions of the SMAIS-ULM in consecutive time-frames. For instance, in table P12, [redacted] respondents' score at the FU 5 visit represented an increase compared to their previously submitted score, but this previous score may have been entered at baseline, FU 1, FU 2, FU 3 or FU 4.

Table P12. Type II and III non-sitters. Change in total (adjusted) score) from the previous SMAIS-ULM questionnaire submitted by each respondent, according to visit window.

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	Increase n (%)	Same n (%)	Decrease n (%)	Mean score change from previous submission
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All (without N/A)	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

*This was the first SMAIS-ULM submission by this patient while on treatment, so there is no point of comparison to a previous submission.

Table P13. Type II and III sitters. Change in total (adjusted) score from the previous SMAIS-ULM questionnaire submitted by each respondent, according to visit window.

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	Increase n (%)	Same n (%)	Decrease n (%)	Mean change from previous submission
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All (without N/A)	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

*This was the first SMAIS-ULM submission by this patient while on treatment, so there is no point of comparison to a previous submission.

Table P14. Type II and III walkers. Change in total (adjusted) score from the previous SMAIS-ULM questionnaire submitted by each respondent, according to visit window.

Clinic visit timepoint (time from baseline +/- 3 months)	Responses n	N/A* n (%)	Increase n (%)	Same n (%)	Decrease n (%)	Mean change from previous submission
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
[illegible]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]
All (without N/A)	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]	[redacted]

*This was the first SMAIS-ULM submission by this patient while on treatment, so there is no point of comparison to a previous submission.

Fig. P4. Type II and III non-sitters, sitters, and walkers. Proportion of change in score of SMAIS-ULM responses compared to patient's previous SMAIS-ULM response. Excludes patient's first response (N/A).