

Psychometric Analysis of the Patient-Reported Plexiform Neurofibromas Quality of Life Measure Using KOMET Study Data

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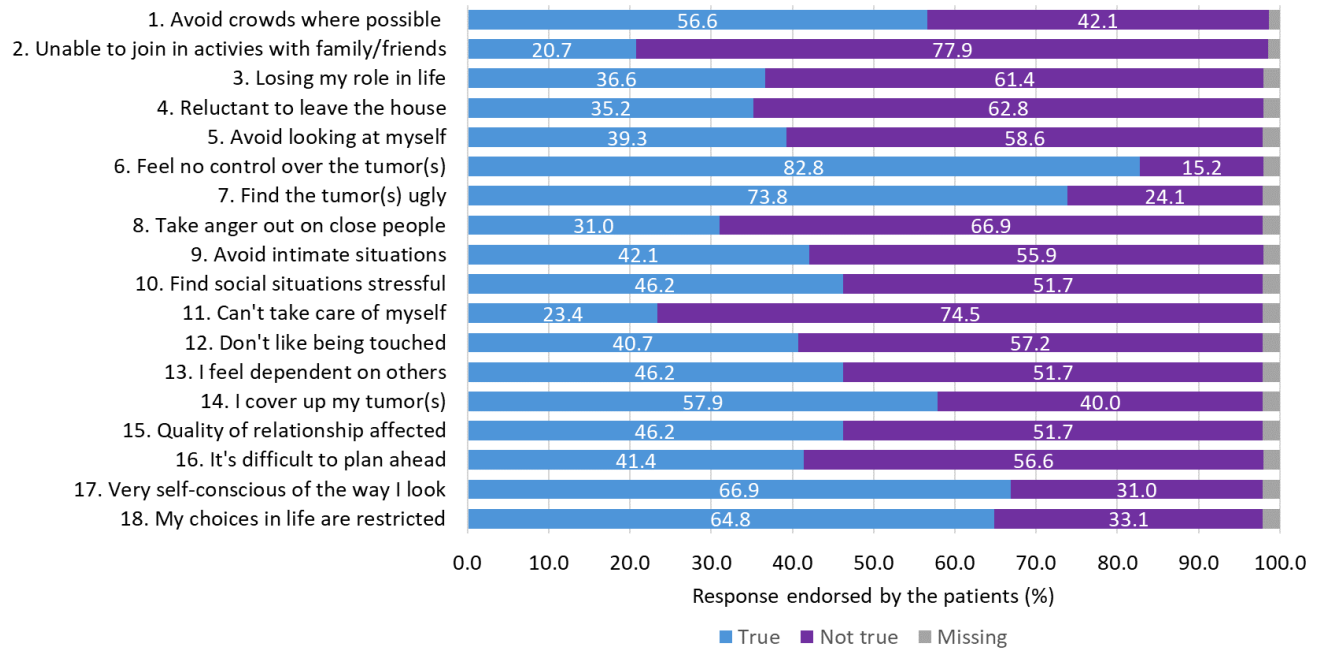
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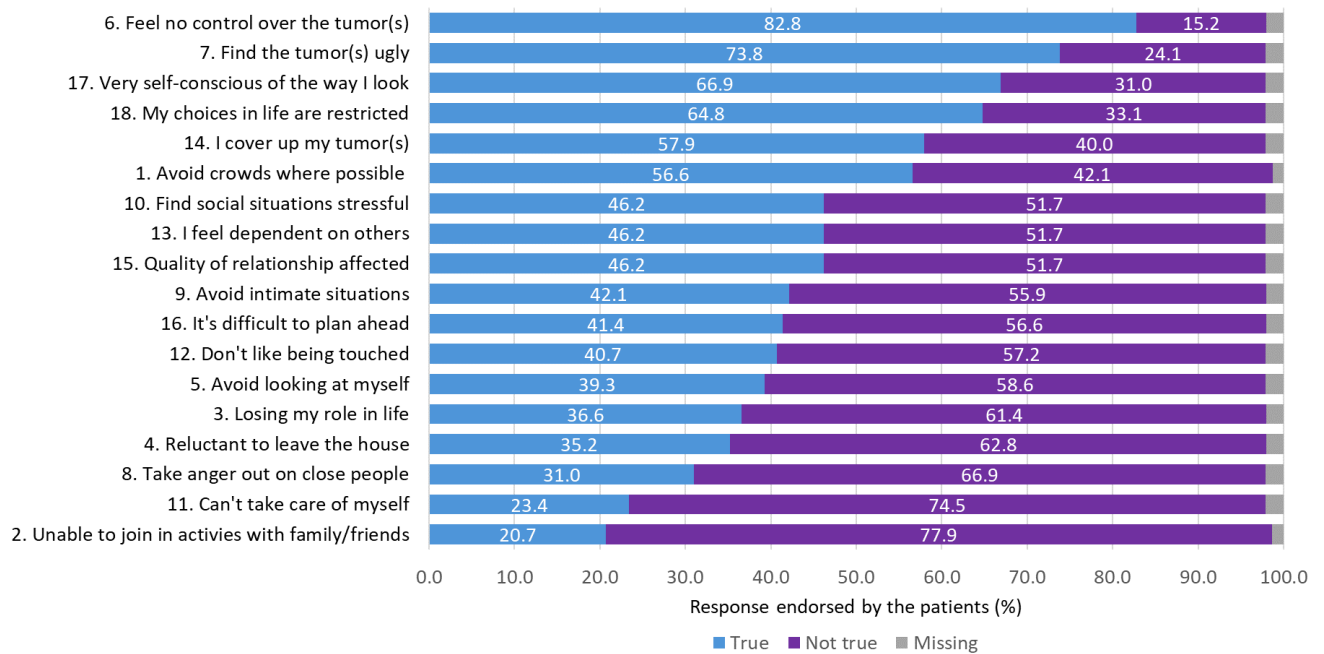
Supplementary Materials

Supplementary Figure S1. Distribution of PlexiQoL item response at baseline ordered by (a) item placement in the questionnaire and, (b) patient response (FAS, N = 145)

(a)



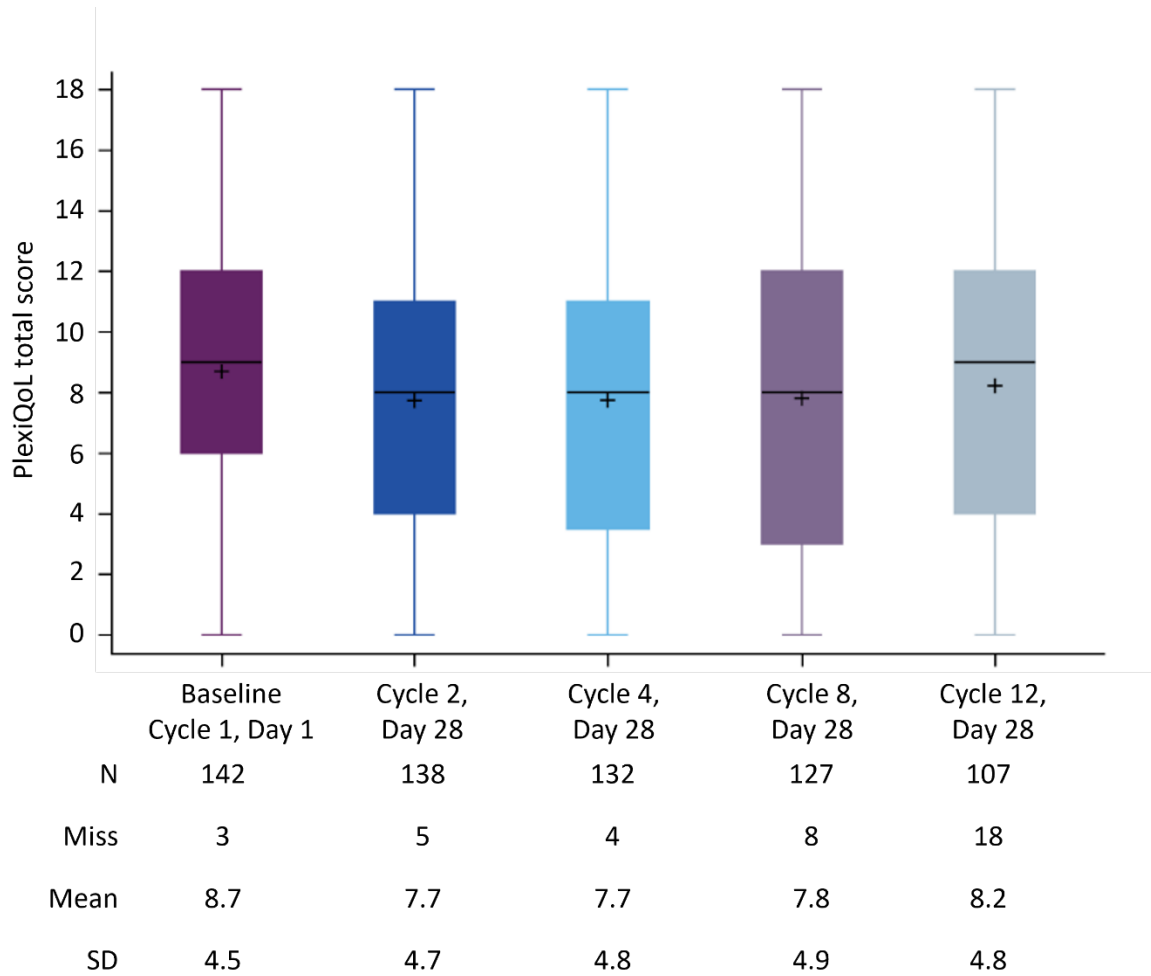
(b)



In panel (a), items are ordered according to original placement in the PlexiQoL. In panel (b), items are ordered from top to bottom according to the item location estimated by the Rasch model (i.e., average of the item threshold) to display the distribution according to patient response to the PlexiQoL.

FAS, full analysis set; PlexiQoL, plexiform neurofibromas quality of life questionnaire.

Supplementary Figure S2. Distribution of the PlexiQoL total score over the first 12 cycles



The line on each plot represents the median value; + represents the mean value. The different colors on the chart are used to distinguish between Baseline, Cycle 2, Cycle 4, Cycle 8, and Cycle 12.

Miss, number of missing values; N, number of available values; PlexiQoL, plexiform neurofibromas quality of life questionnaire; SD, standard deviation.

Supplementary Table S1. Test–retest reliability of the PlexiQoL total score (FAS, N = 145)

| ICC type (95% CI) | Baseline to Cycle 2 | | | Cycle 8 to Cycle 12 | | |
|--|-------------------------|-----------------------------------|---------------------------------------|-------------------------|-----------------------------------|---------------------------------------|
| | FAS N = 145 | Stable (EQ-5D-5L items) N = 18 | Stable (EQ-5D-5L VAS score) N = 36 | FAS N = 129 | Stable (EQ-5D-5L items) N = 28 | Stable (EQ-5D-5L VAS score) N = 54 |
| ICC(1): one-way random, absolute agreement, single rater | 0.749 (0.668, 0.812) | 0.940 (0.851, 0.977) | 0.808 (0.658, 0.897) | 0.832 (0.770, 0.878) | 0.848 (0.701, 0.926) | 0.871 (0.788, 0.923) |
| ICC(C,1): two-way random/mixed, consistency, single rater | 0.764 (0.687, 0.824) | 0.938 (0.842, 0.976) | 0.828 (0.689, 0.909) | 0.834 (0.773, 0.880) | 0.866 (0.731, 0.936) | 0.871 (0.787, 0.923) |
| ICC(A,1): two-way random/mixed, absolute agreement, single rater | 0.752 (0.662, 0.819) | 0.940 (0.849, 0.977) | 0.810 (0.642, 0.901) | 0.833 (0.771, 0.879) | 0.850 (0.683, 0.930) | 0.871 (0.788, 0.923) |

ICC(A,1) = two-way random/mixed, absolute agreement, single rater; ICC(C,1) = two-way random/mixed, consistency, single rater – labeled according to the convention from McGraw KO and Wong SP. *Psychological Methods* 1996;1:30.

Two definitions used for stability: same responses to all EQ-5D-5L items and change in EQ-5D-5L VAS score of <7.

CI, confidence interval; EQ-5D-5L, EuroQol 5-dimension 5-level questionnaire; FAS, full analysis set; ICC, intraclass correlation coefficient; PlexiQoL, plexiform neurofibromas quality of life questionnaire; VAS, visual analog scale.

Supplementary Table S2. Description of the change in PlexiQoL total score from baseline at Cycle 12 across categories of change in EQ-5D-5L VAS score (FAS, N = 145)

| Variable | Change in EQ-5D-5L VAS score from baseline to Cycle 12 | | |
|-------------|--|--|---|
| | Worsened (change ≤ -10) N = 15 | Stable ($-10 < \text{change} < 10$) N = 50 | Improved (change ≥ 10) N = 39 |
| N (missing) | 15 (0) | 50 (0) | 39 (0) |
| Mean (SD) | 0.93 (3.83) | -0.36 (3.35) | -0.64 (3.29) |
| Median | 0.00 | 0.00 | -1.00 |
| Q1, Q3 | -2.00, 4.00 | -3.00, 2.00 | -3.00, 2.00 |
| P10, P90 | -4.00, 7.00 | -4.00, 5.00 | -5.00, 4.00 |
| Min, Max | -5.00, 8.00 | -8.00, 6.00 | -7.00, 7.00 |

EQ-5D-5L, EuroQol 5-dimension 5-level questionnaire; FAS, full analysis set; max, maximum; min, minimum; P10, 10th percentile; P90, 90th percentile; PlexiQoL, plexiform neurofibromas quality of life questionnaire; Q1, first quartile; Q3, third quartile; SD, standard deviation; VAS, visual analog scale.