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Introduction and objectives

- Myasthenia Gravis (MG) is a rare, chronic, autoimmune neuromuscular disease which causes debilitating and fluctuating muscle weakness ¹.
- It affects vision, swallowing, speech, mobility, dexterity and respiratory function, thus adversely impacts patients' health-related quality of life (HRQoL)²⁻⁵.
- The objective of the present analysis was to compare utility values of adult MG patients with those of the general population, collected via the five-level version of EQ-5D (EQ-5D-5L) and the Health Utility Index (HUI3).

Methods

- The MyRealWorld-MG (MRW-MG) study is a digital, observational, multi-country study (US, UK, Canada, Italy, Germany, Spain, Japan) among adult MG patients ⁶. Patients entered personal and disease characteristics via a smartphone application and filled in the EQ-5D-5L ⁷ and the HUI3 ⁸.
- The POPUP observational study collected similar data and was conducted in the US, UK, Canada, Belgium, Italy, Germany, Netherlands and Spain. POPUP enrolled members of the general public and national samples were representative by age, gender, education and region.
- Participants from both studies were asked to complete the Myasthenia Gravis Activities of Daily Living (MG-ADL) questionnaire; the MRW-MG patients completed it as a measure of disease severity, POPUP participants completed it as a measure of level of functionality and health status without any reference to MG, as well as for comparative purposes. Data on the level sum score of all MG-ADL items is used in the present analysis.
- For the analysis and between the two groups, POPUP data were age-gender matched to the MRW-MG population.
- EQ-5D-5L utility values by country were estimated based on the country-specific value sets to the respondents from that country for POPUP; for the MRW-MG study the national value sets have been applied each time to the entire patient population. The overall utilities are an average of the utilities obtained by applying each patient's country-specific value sets to his/her responses. HUI3 utility values were estimated based on the only available Canadian value set.

Results

1. Population characteristics

- The MRW-MG study enrolled 1,859 adult patients.
- The POPUP study enrolled 9,000 participants and the sample was representative of the general population in each country.
- The demographics and characteristics of the population included in the two studies are presented in Table 1.
 - The MG-ADL and EQ VAS scores are presented as a measure of the level of functionality and overall health of the study populations in the two groups
 - For the MRW-MG study population, the MG-ADL score is also an index of disease severity (the higher the score, the more severe the disease, as follows: 0-6: mild; 7-14: moderate; ≥15: severe).

Table 1. Demographics & characteristics of the participants of the POPUP and MRW-MG studies

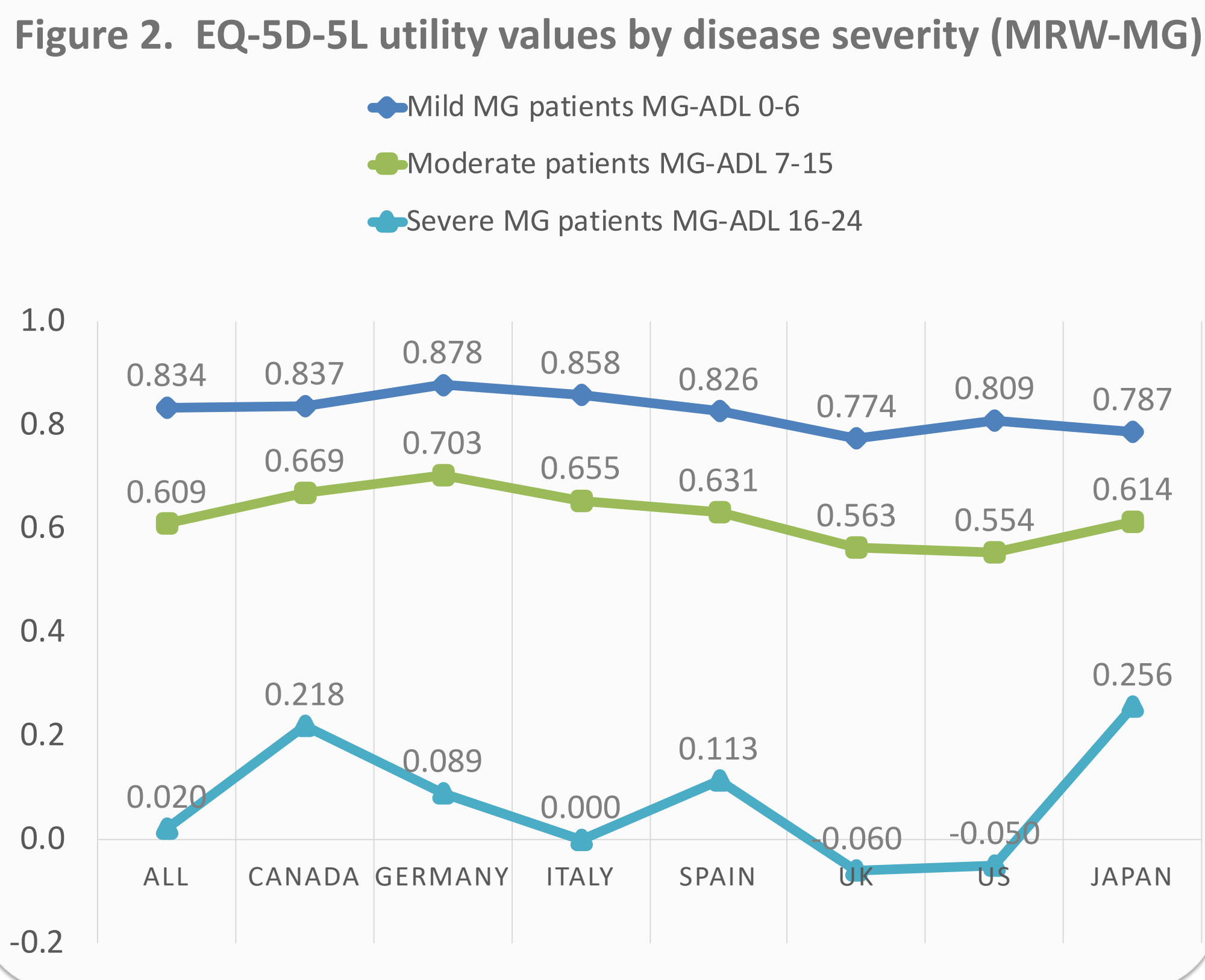
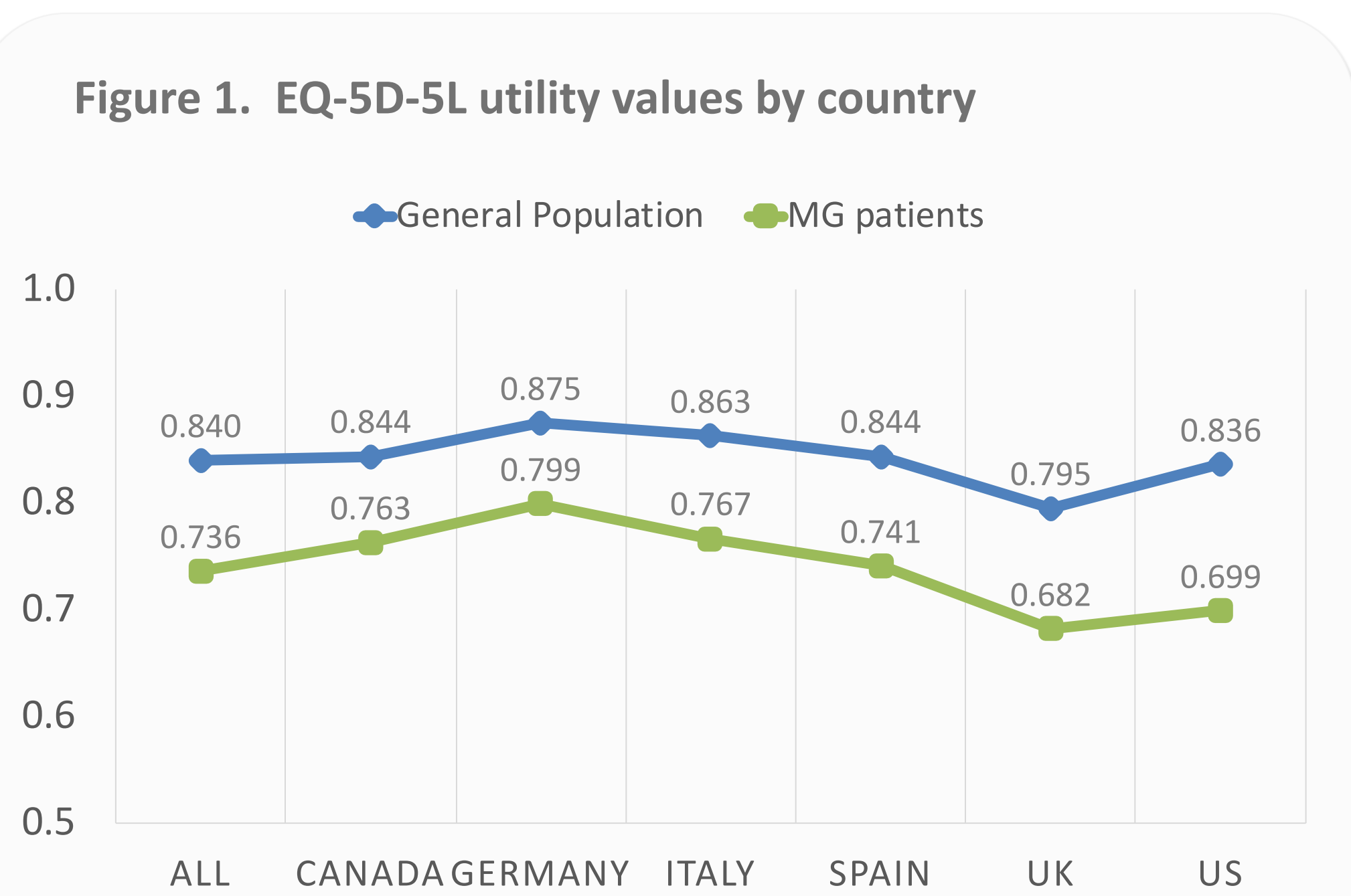
	POPUP (N=9,000)	MRW-MG (N=1,859)
Gender (% Female)	51.2%	68.8%
Age distribution		
18-34	27.6%	16.5%
35-54	36.9%	44.5%
55+	35.5%	39.0%
Living situation		
At home without help from a caregiver	70.5%	52.8%
At home with help from a caregiver	3.1%	5.0%
With a family member	26.0%	36.3%
In a nursing home	0.3%	0.1%
In a long-term care rehabilitation facility	0.1%	0.4%
Activities of daily living/ Disease severity		
MG-ADL score*: 0-6	95.5%	59.0%
7-14	3.7%	40.0%
≥ 15	0.8%	1.0%
VAS Mean (Std)	75.7 (17.4)	61.4 (22.3)

* Higher MG-ADL score indicate higher difficulty in performing the activities of daily leaving.

Note: Table 1 presents the unadjusted data on POPUP study participants. For the analysis and comparison with the MRW-MG, data from the POPUP study were weighted by MRW-MG age and gender distribution. Differences between adjusted and unadjusted data were minor.

2. EQ-5D-5L utility values

- Mean utility values measured via the EQ-5D-5L were consistently lower in the MRW-MG patient population than in the general population of the POPUP study (Figure 1).
- This was consistent across all country-specific value sets, with the utility decrements ranging between 0.076 (when applying the German value set) and 0.137 (with the US value set).
- For the entire population, the utility difference between the MRW-MG and the general population was -0.104, which is more than the minimally important difference (MID) of 0.037 to 0.69 for EQ-5D-5L ⁹
- EQ-5D-5L utilities were associated with disease severity in the MRW-MG patient population (Figure 2).
- When applying the UK (crosswalk) value set, utilities ranged from 0.774 to -0.060 for mild and severe MG, respectively.
- Overall, utility values in the severe patient population were very low, even equal to (when applying the Italian value set) or below 0 (when applying UK and the US value sets)..



3. HUI3 utility values

- The HUI3 utility values in the POPUP general population were estimated at 0.747, much higher than the respective value for the total MRW-MG patient population (0.483).
- Therefore, MG is associated with a significant utility decrement of 0.264, which is much higher (8-fold) than the MID for HUI3 (0.032) ⁸.
- This significant utility decrement in the HUI measurement is attributed to the fact that HUI3 explicitly captures the disease impact on aspects that are severely compromised in MG patients, such as vision (Figure 3), speech (Figure 4), dexterity (Figure 5) and ambulation (Figure 6).

Figure 3. HUI3: Vision

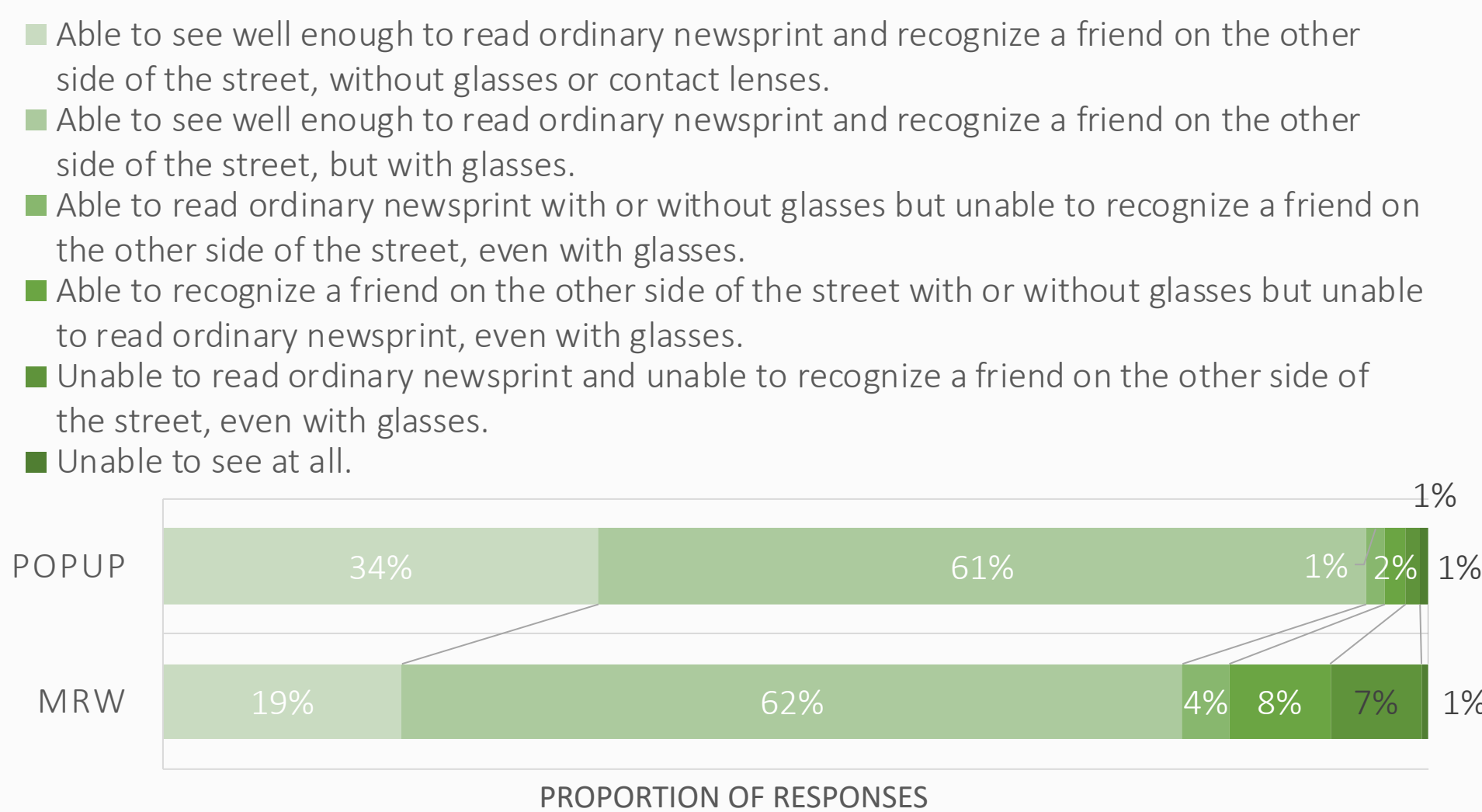


Figure 4. HUI3: Speech

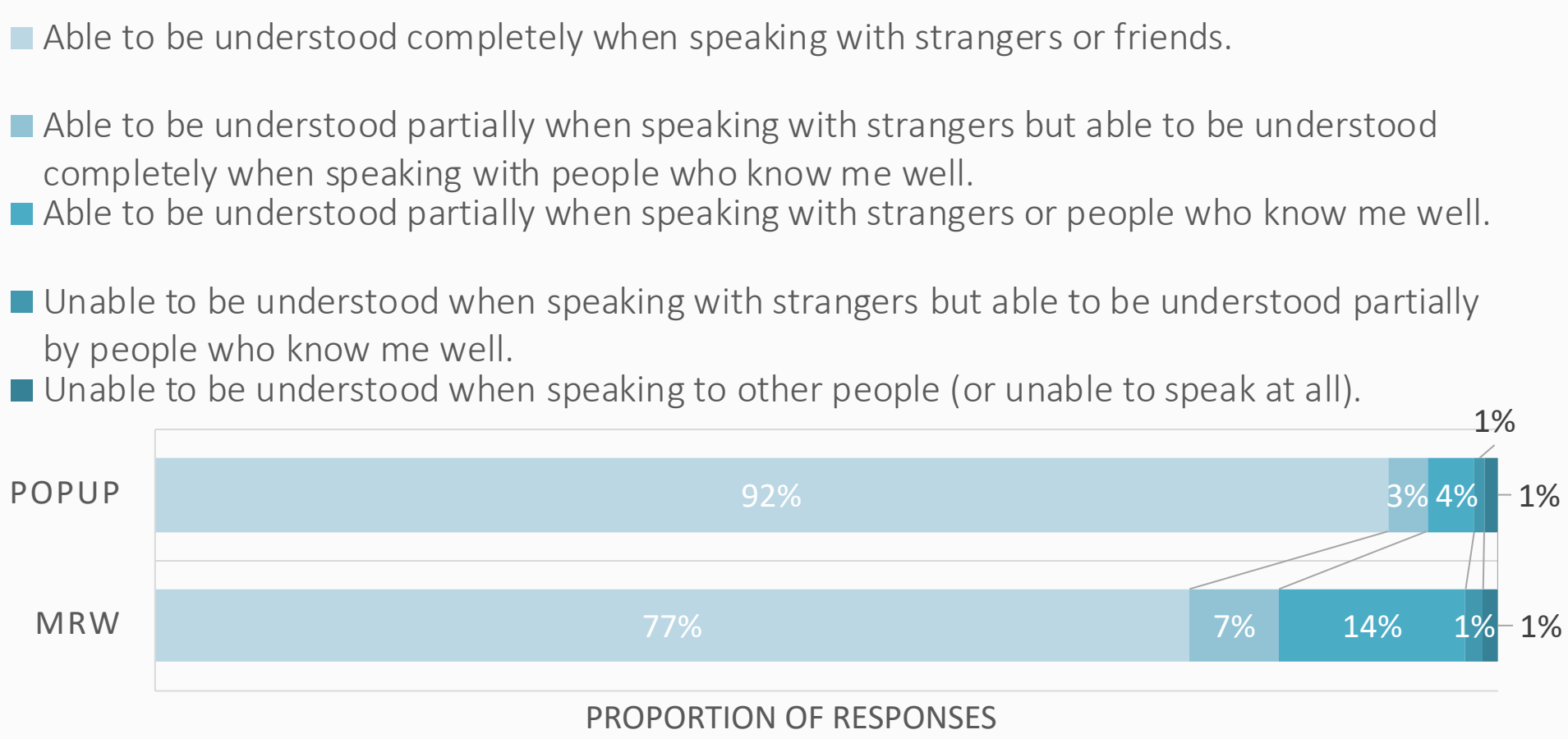


Figure 5. HUI3: Dexterity

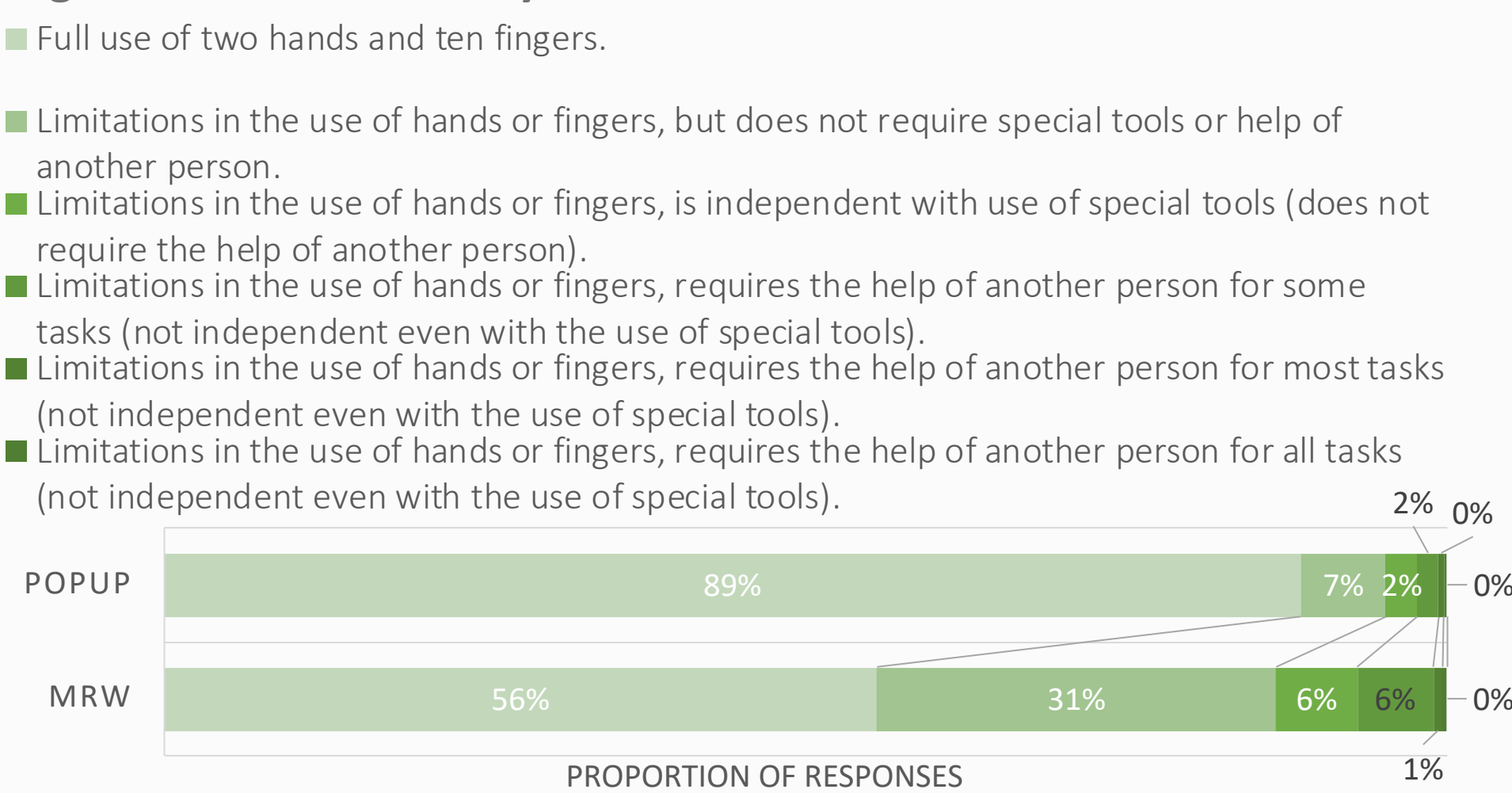
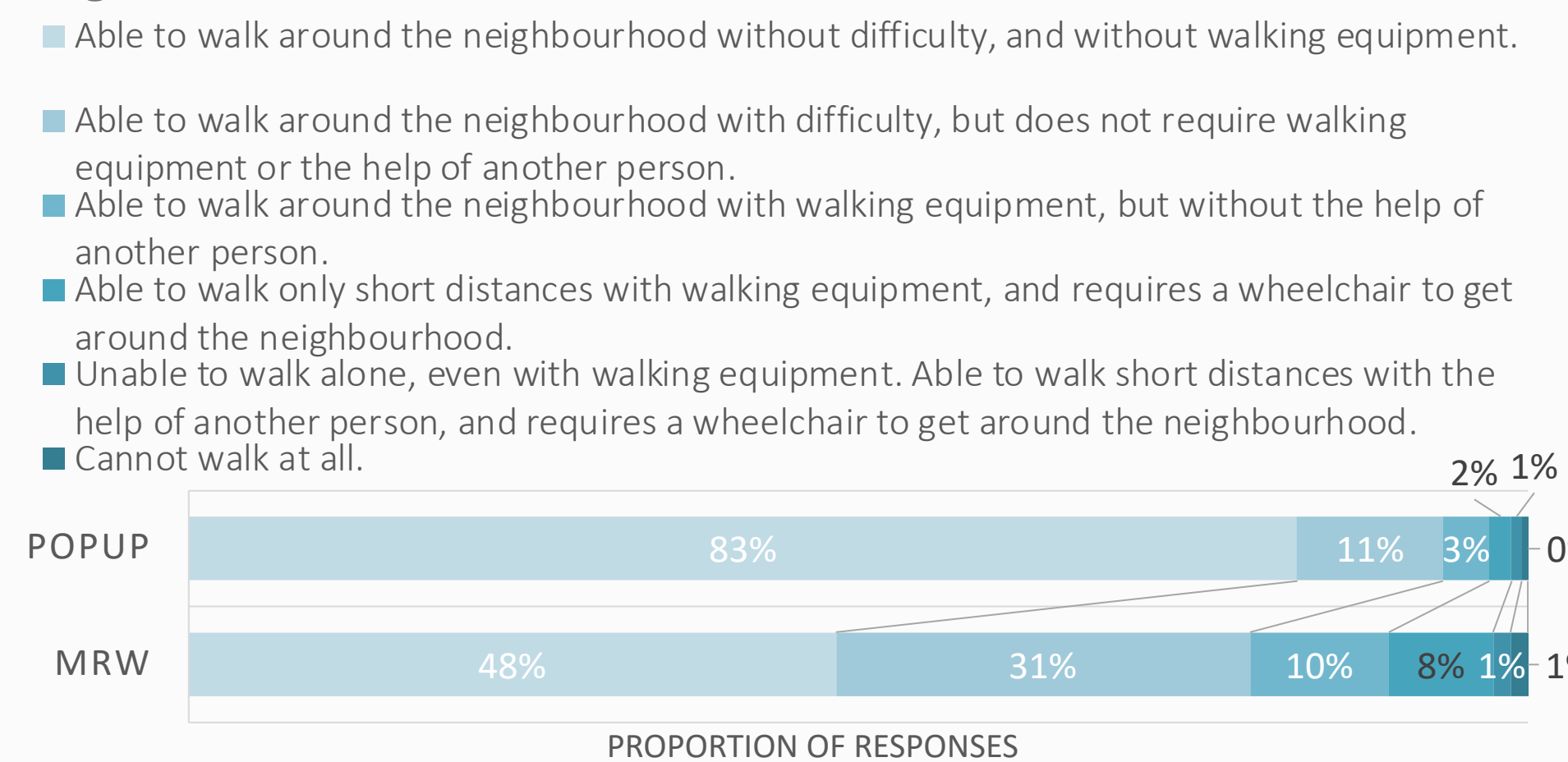


Figure 6. HUI3: Ambulation



Discussion & Conclusions

- The very low EQ-5D-5L utility values of the severe MG patient population (as low as or even below zero), show that severe MG patients experience health states regarded as equivalent to/ worse than a state that is as bad as being dead ⁷.
- The HUI3 instrument appears to have recorded a larger impact of MG on patients' utility than the EQ-5D-5L. This is attributed to the fact that HUI3 classification system comprises of eight attributes (Vision, Speech, Ambulation, Dexterity, Emotion, Cognition and Pain – each with 5 or 6 levels of ability/disability ⁸), all of which are severely affected by MG.

Overall, adult MG patients experience numerically and meaningfully lower health utility values than an age-gender matched general population sample.

References

- Gilhus NE et al., Nat Rev Dis Primers, 2019; 5:30
- <https://www.nhs.uk/conditions/myasthenia-gravis/>
- Behr and Silvestri, Neurol. Clin., 2018; 36:2
- Boldingh MI et al., Health Qual Life Outcomes, 2015; 13:115.
- Gelinas D. et al., Journal of the Neurological Sciences, 2022; 437
- Berrih-Aknin S et al., BMJ Open, 2021;11
- www.euroqol.org
- Horsman J et al., Health and Quality of Life Outcomes. 2003;1:54.
- McClure NS et al., Value in Health 2017; 20

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Abbreviations MG, myasthenia gravis; MRW-MG, MyRealWorld-MG; HRQoL: health-related quality of life; EQ-5D-5L, five-level EuroQol five-dimensional questionnaire; HUI: health utility index; MG-ADL: MG-Activities of daily living; Std, standard deviation; MID: minimally important difference.