Association Between Health-Related Quality of Life and Requirement for Non-Professional Care in Adults with Rare Diseases: A Real-World Study

Ben Johnson[1], James Piercy[2], Jonathan DeCourcy[2], Giorgio Castellano[2], Halima Iqbal[2], Gregor Gibson[2], Angela Williams[1]

[1]Kyowa Kirin International, Marlow, UK; [2]Adelphi Real World, Bollington, UK.

OBJECTIVE

- This study aimed to assess whether adult patients' health-related quality of life (HRQoL) is associated with the requirement for non-professional care across five rare diseases.

CONCLUSIONS

- Patients requiring non-professional caregivers reported significantly worse scores in each of the five EQ-5D dimensions.
- Multivariate regression results showed a significant, negative association between patients' EQ-5D utility and requirement for non-professional care, indicating that improvements to HRQoL in individuals with rare diseases may lead to a reduced burden on family and friends.

Poster presented at ISPOR EU 2023 - Corresponding author email address: Ben.Johnson@kyowakirin.com

INTRODUCTION

- Rare diseases are often associated with a considerable humanistic and productivity burden for patients and their caregivers.[1, 2]
- Limited treatment options, reduced access to information, and practical difficulties such travelling further to specialists all contribute to the higher emotional and physician burden of patients with rare diseases, and those who care for them, when compared to more common conditions.[3, 4]
- Non-professional care is common, typically provided by family and friends.[3, 5]

METHODS

- We analysed real-world data from Adelphi Disease Specific Programmes (DSPs) for five rare diseases: Amyotrophic Lateral Sclerosis (ALS), Graft-Versus-Host Disease (GVHD), Huntington's Disease (HD), Myasthenia Gravis (MG), Progressive Supranuclear Palsy (PSP).
- Data were collected in France, Germany, Italy, Spain, the UK and the US from July 2017 to March 2021.
- The DSP methodology has been previously published and validated.[6, 7]
- Physicians provided cross-sectional data on patients' characteristics and non-professional care requirements whilst patients self-reported HRQoL via the EQ-5D-5L.[8]
- Responses to the EQ-5D-5L were mapped to the EQ-5D-3L and valued using a UK scoring algorithm to produce utilities.[9, 10]
- Bivariate analysis (using chi squared, T-tests, Mann-Whitney, Fisher's exact test) assessed differences in patients' characteristics and EQ-5D utility for those with and without a non-professional caregiver.
- Multivariable logistic regression assessed the relationship between patients' EQ-5D utility and presence of a non-professional caregiver, controlling for patients' characteristics (age, sex, geographic location, and disease).

RESULTS

- The analysis included 823 patients with a mean (standard deviation [SD]) age of 55.8 (14.5) years; 58.6% were male; 23.0% from the USA (**Table 1**).
- 12.0%, 28.6%, 5.7%, 37.9%, and 15.8% of patients had ALS, GVHD, HD, MG, and PSP, respectively (**Table 2**).
- Non-professional caregivers were reported for 49.8% of patients (range 37.8% MG – 60.8% PSP).
- Patients had a mean (SD) EQ-5D utility of 0.64 (0.24) (**Table 3**).
- Bivariate analysis showed patients with at least one physician-reported non-professional caregiver were older (57.7 versus 53.9; p=0.0001) and had a worse quality of life using the EQ-5D-5L utility (0.56 versus 0.72; p<0.0001) than patients without a non-professional caregiver (**Table 3**).
- Patients without a non-professional caregiver reported no problems or slight problems less frequently in each of the five dimensions of the EQ-5D (Anxiety / Depression, Pain / Discomfort, Usual activities, Self-care and Mobility, all p<0.0001) than those with at least one non-professional caregiver (**Figure 1**).

Table 1. Sample breakdown by country of respondent

Country	Total N=823	No non-professional caregiver(s) N=413 (50.2%)	At least one non-professional caregiver(s) N=410 (49.8%)	*p*-value
France, N (%)	96 (11.7)	41 (9.9)	55 (13.4)	0.1291
Germany, N (%)	206 (25.0)	107 (25.9)	99 (24.1)	0.5740
Italy, N (%)	145 (17.6)	69 (16.7)	76 (18.5)	0.5223
Spain, N (%)	151 (18.3)	67 (16.2)	84 (20.5)	0.1259
UK, N (%)	36 (4.4)	17 (4.1)	19 (4.6)	0.7364
USA, N (%)	189 (23.0)	112 (27.1)	77 (18.8)	0.0048

UK; United Kingdom, USA; United States of America.

Table 2. Sample breakdown by patient's condition

Condition	Total N=823	No non-professional caregiver(s) N=413	At least one non-professional caregiver(s) N=410	*p*-value
ALS, N (%)	99 (12.0)	40 (9.7)	59 (14.4)	0.8817
GVHD, N (%)	235 (28.6)	105 (25.4)	130 (31.7)	0.0074
HD, N (%)	47 (5.7)	23 (5.6)	24 (5.9)	0.0536
MG , N (%)	312 (37.9)	194 (47.0)	118 (28.8)	<0.0001
PSP, N (%)	130 (15.8)	51 (12.3)	79 (19.3)	0.0418

ALS; Amyotrophic Lateral Sclerosis, GVHD; Graft versus host disease, HD; Huntington's disease, MG; Myasthenia Gravis, PSP; Progressive Supranuclear Palsy.

Figure 1. EQ-5D dimensions for patients with and without non-professional care

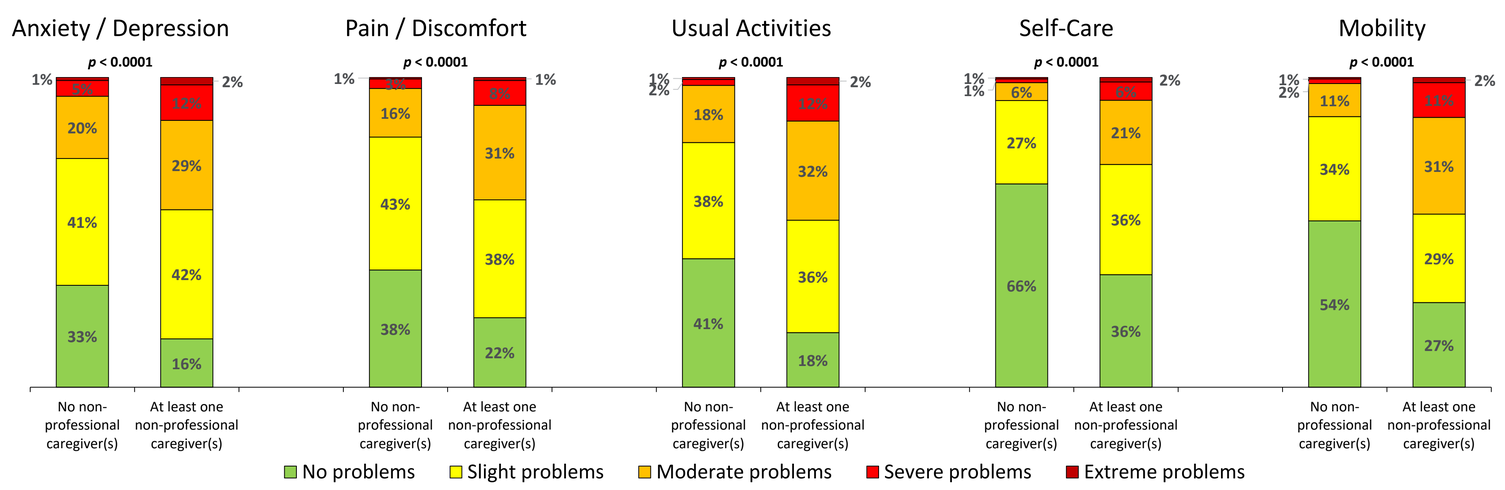

Figure 2. Predictors of non-professional caregiving

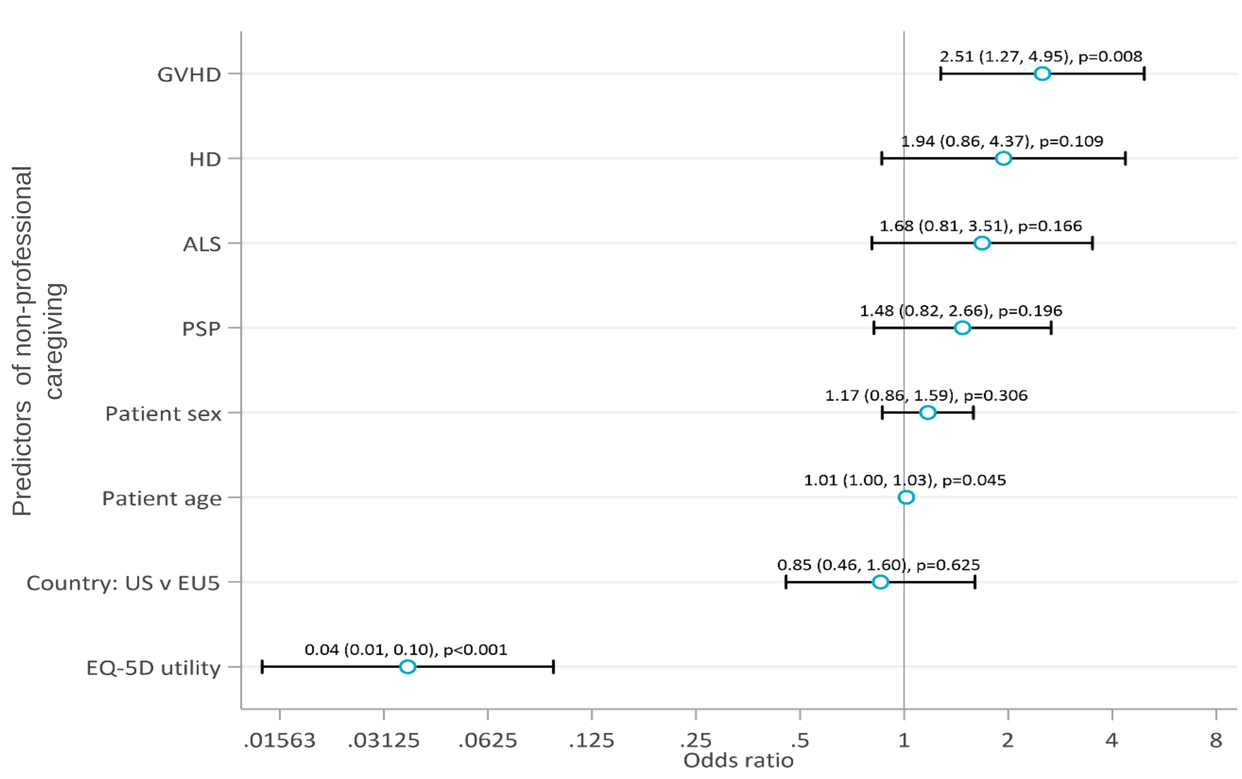

Table 3. Patient demographics and EQ-5D-5L utility score

	Total N=823	No non-professional caregiver(s) N=413	At least one non-professional caregiver(s) N=410	*p*-value
Gender; male, N (%)	482 (58.6)	230 (55.7)	252 (61.5)	0.1036
Age; years, Mean (SD)	55.8 (14.5)	53.9 (14.9)	57.7 (13.8)	0.0001
Time since diagnosis of condition; months, Mean (SD)	25.9 (39.2)	28.0 (44.0)	24.0 (33.7)	0.1668
EQ-5D-5L utility, Mean (SD)	0.64 (0.24)	0.72 (0.20)	0.56 (0.26)	<0.0001

SD; Standard deviation.

- Multivariable regression showed a significant (p<0.0001) association between EQ-5D utility and presence of a non-professional caregiver (odds ratio [OR] 0.037; 95% confidence intervals [CI] 0.014-0.097), indicating that an increase of 0.1 in EQ-5D utility is associated with a 28% decrease in the odds of receiving non-professional care (**Figure 2**).
- Additionally, older patients, and those with GVHD (using MG as a reference category), were significantly more likely to receive non-professional care (**Figure 2**).

LIMITATIONS

- Presence of non-professional caregivers was physician reported and relied upon physician awareness of care requirements.
- DSPs may not be fully representative of the wider rare disease population: participation was influenced by willingness to complete the survey, and patients consulting with their HCP more frequently may be overrepresented in the sample.
- Quality of data relies on physicians' and patients' ability to recall and accurately report information.

ACKNOWLEDGEMENTS

The authors would like to thank the physicians and patients for taking part in this study and providing the information included.

DISCLOSURES

- BJ and AW are employees of Kyowa Kirin International.
- JP, JdC, GC, HI and GG are employees of Adelphi Real World.
- Adelphi Real World received funding from Kyowa Kirin for this research. Each DSP is an Adelphi Real World product. Kyowa Kirin subscribed to the datasets and did not influence the original surveys through either contribution to the design of questionnaires or data collection.

REFERENCES

1. Pillas, D., Klein, A., Gasalla, T., Avbersek, A., Thompson, A., Wright, J., Mellor, J. and Scowcroft, A. (2022). The Burden of Progressive Supranuclear Palsy on Patients, Caregivers, and Healthcare Systems by PSP Phenotype: A Cross-Sectional Study. Frontiers in Neurology, [online] 13, p.821570. doi:https://doi.org/10.3389/fneur.2022.821570.
2. Ignatova, V., Kostadinov, K., Vassileva, E., Muradyan, N., Stefanov, G., Iskrov, G. and Stefanov, R. (2022). Socio-Economic Burden of Myasthenia Gravis: A Cost-of-Illness Study in Bulgaria. Frontiers in Public Health, 10. doi:https://doi.org/10.3389/fpubh.2022.822909.
3. Mcmullan, J., Lohfeld, L. and McKnight, A.J. (2022). Needs of informal caregivers of people with a rare disease: a rapid review of the literature. BMJ Open, [online] 12(12), p.e063263. doi:https://doi.org/10.1136/bmjopen-2022-063263.
4. Sandilands, K., Williams, A. and Rylands, A.J. (2022). Carer burden in rare inherited diseases: a literature review and conceptual model. Orphanet Journal of Rare Diseases, 17(1). doi:https://doi.org/10.1186/s13023-022-02561-w.
5. Valcárcel-Nazco, C., Ramallo-Fariña, Y., Linertová, R., Ramos-Goñi, J.M., García-Pérez, L. and Serrano-Aguilar, P. (2022). Health-Related Quality of Life and Perceived Burden of Informal Caregivers of Patients with Rare Diseases in Selected European Countries. International Journal of Environmental Research and Public Health, [online] 19(13), p.8208. doi:https://doi.org/10.3390/ijerph19138208.
6. Anderson P, Benford M, Harris N, Karavali M, Piercy J. Real-world physician and patient behaviour across countries: Disease-Specific Programmes - a means to understand. Curr Med Res Opin. 2008 Nov;24(11):3063-72. doi: 10.1185/03007990802457040. Epub 2008 Oct 2. PMID: 18826746.
7. Babineaux SM, Curtis B, Holbrook T, Milligan G, Piercy J. Evidence for validity of a national physician and patient-reported, cross-sectional survey in China and UK: the Disease Specific Programme. BMJ Open. 2016;6(8):e010352. Published 2016 Aug 16. doi:10.1136/bmjopen-2015-010352
8. EuroQoL. EQ-5D-5L. https://euroqol.org/eq-5d-instruments/eq-5d-5l-about/ Accessed 31 October 2022.
9. Hernández-Alava M, Pudney S. EQ5Dmap: A Command for Mapping between EQ-5D-3L and EQ-5D-5L. The Stata Journal. 2018;18(2):395-415. https://doi.org/10.1177/1536867X1801800207
10. Hernández Alava M, Pudney S, Wailoo A. Estimating the Relationship Between EQ-5D-5L and EQ-5D-3L: Results from a UK Population Study. Pharmacoeconomics. 2023 Feb;41(2):199-207. doi: 10.1007/s40273-022-01218-7. Epub 2022 Nov 30. PMID: 36449173; PMCID: PMC9883358.