Comparison of Rare Disease Patient and Caregiver Disease Burden in Terms of Economic, Social, and Interpersonal Impact and Impact on Quality Of Life: Results from an International Survey

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BACKGROUND

- The burden of rare diseases (RDs) affects not only patients but also families and caregivers, bringing significant social, emotional, and financial challenges.
- Around 300 million people globally are impacted by RDs, most of which are hereditary and appear in childhood.
- Caregivers often face financial strain, with one parent typically leaving work to provide care, further increasing stress.
- Social isolation is common, as families receive little support, and caregivers take on multiple roles due to a lack of medical resources.
- Emotional burdens like anxiety and depression are frequent, while patients themselves often feel isolated from their peers.

OBJECTIVE

- To understand the relative impact of a rare disease (RD) on patients and/or caregivers socially, economically, interpersonally and on their quality of life (QoL).

METHODS

- An international survey with 800 participants was conducted across Germany, United Kingdom (UK), Spain and the United States (U.S), using Rare Patient Voice online patient panels.
- RD patient & caregiver opinions about their experience living with RD, including the impact of RD, were gathered using an online survey.
- All participants were sourced from the Rare Patient Voice panels and outreach program. Participants were eligible to participate if they had a RD, were over 18y and from one of the 4 countries mentioned above. Adult caregivers of patients with a RD condition were included as well.
- A combination of qualitative and quantitative data were collected from participants, using an online survey. The quantitative data was analyzed using SPSS , while the qualitative data (verbatims from respondents) was analyzed using the MAXQDA software.

CONCLUSION

- Overall, RD severely impacts patients and their families in multiple aspects of their life, including their economic, social and personal domains. Disease burden varied, depending on whether the respondent was a RD patient, caregiver, or RD patient who was also a caregiver of another RD patient. More social and economic support networks for families and patients with RD is needed, based on their personal situation, to alleviate their disease burden.

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RESULTS

- Survey participants included 100 from Germany, 168 from Spain, 252 from the UK and 280 from the U.S; 78% were RD patients, 15% were caregivers of a RD patient, and 7% represented both patient and caregiver. Over 300 unique RDs were represented within the study cohort.
- More than two-third of RD patients and/or caregivers (76%-79%) experienced economic hardship related to RD disease. (Fig 1)
  - 26%-36% of stakeholders reported spending >10,000 EUR/USD out-of-pocket related to RD medical expenses. (Fig 1)
- Majority of patients (69%), caregivers (72%) and both (77%) felt excluded from social events. (Fig 2)
- Furthermore, majority of patients (86%), caregivers (59%) and both (82%) felt they were a burden to their family and friends. (Fig 2)
- Similarly, majority of patients (71%), caregivers (69%), both (82%) reported that RD severely impacted their QoL. (Fig 3)

Figure 1: Economic hardship of patients, caregivers, and patients who are caregivers

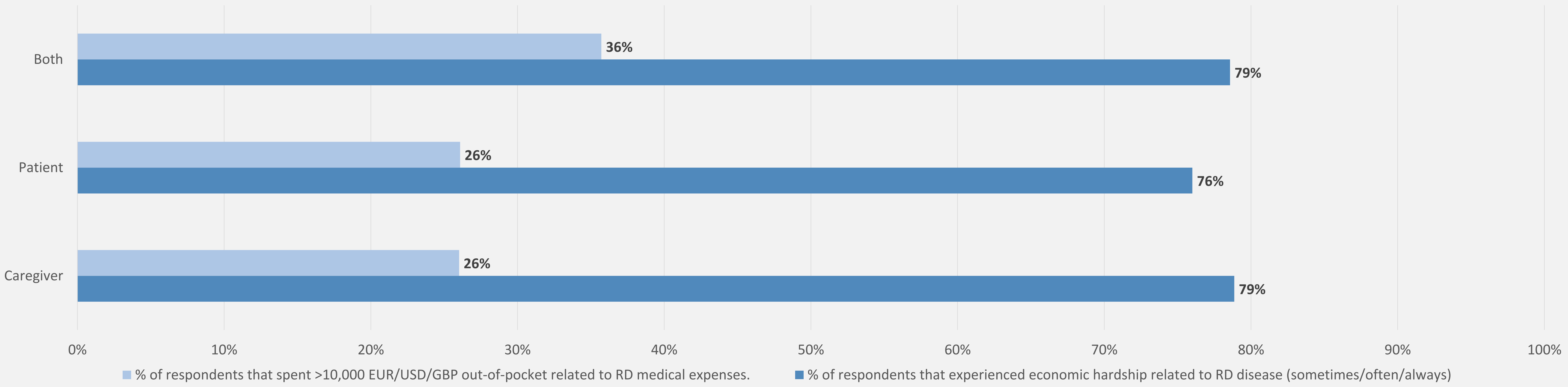


Figure 2: Psycho-social burden experienced by patients, caregivers, and patients who are caregivers

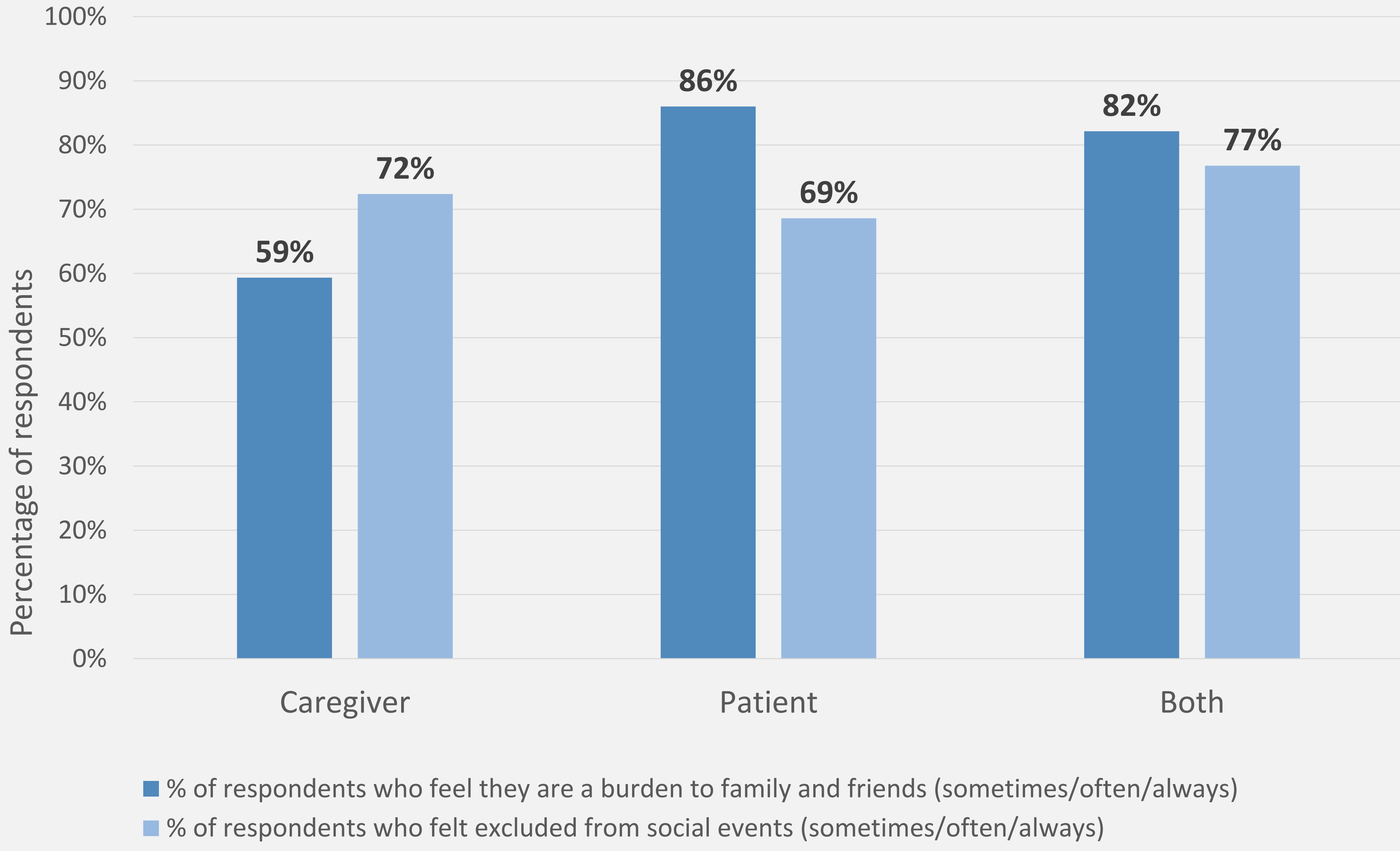
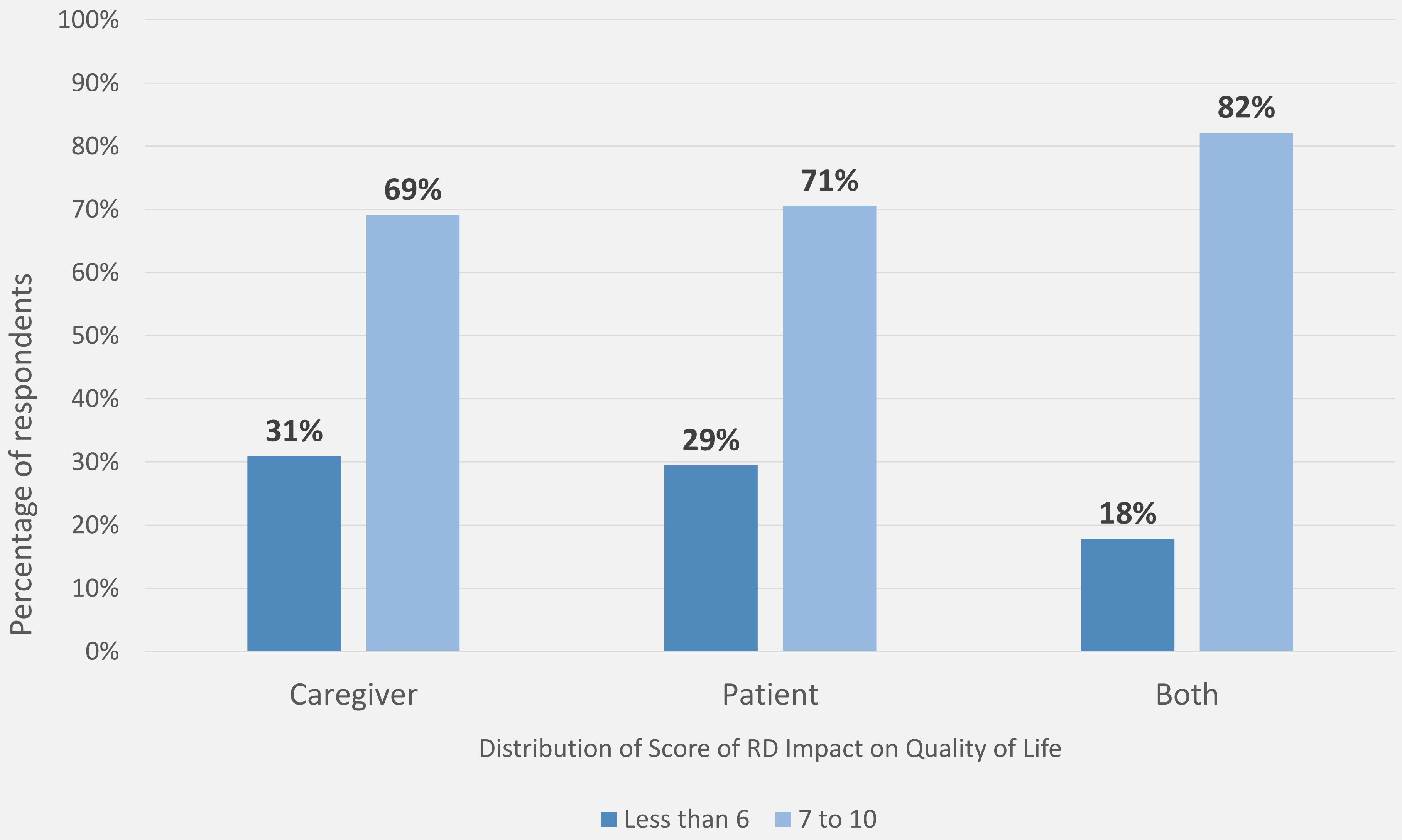


Figure 3: Impact of rare disease on the quality of life



Note: Impact score of 0=least impact, and 10=most impact.

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