

# Impact of Rare Disease on Patients and their Families Economically, Socially, and Interpersonally, and Impact on their Quality Of Life: Results from an International Survey

Siva Narayanan PhD, Avant Health LLC, Bethesda, USA. Wes Michael MBA, Rare Patient Voice, Towson, USA

## 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 impact of a rare disease (RD) on patient and their families 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, rare diseases severely impact patients and their families in multiple aspects of their life, including their economic, personal, and social domains. More social and economic support network for families and patients with rare diseases is needed to alleviate their disease burden.

## References

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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.
- Overall, 77% of participants with a RD experienced economic hardship related to their disease (Fig 1); 27% reported spending >10,000 EUR/USD out-of-pocket related to RD medical expenses.
- Over two-third (71%) reported that RD severely impacted their QoL. (Fig 2)
- Majority (82%) reported that their RD affected the time they spend with friends or family (Fig 3), and 70% of these stakeholders felt excluded from social events. (Fig 4)
- Furthermore, 81% of respondents felt they were a burden to their family and friends (Fig 5), and 84% reported that having an RD impacts the relationship with their family. (Fig 6)

### Rare disease impact on family finances

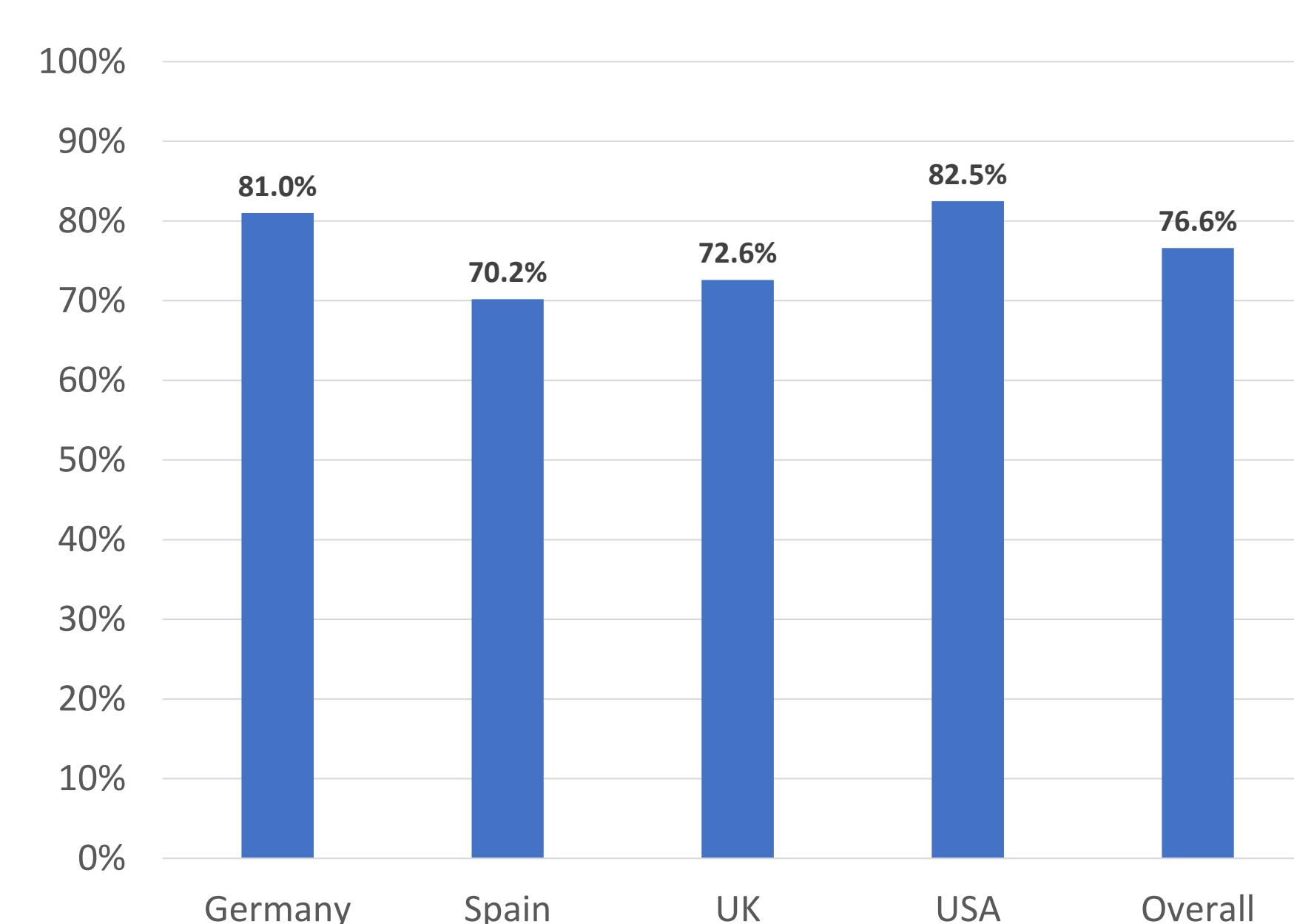


Figure 1. Percentage of respondents reporting somewhat/moderate/severe impact of RD on their family finances

### Rare disease impact on quality of life

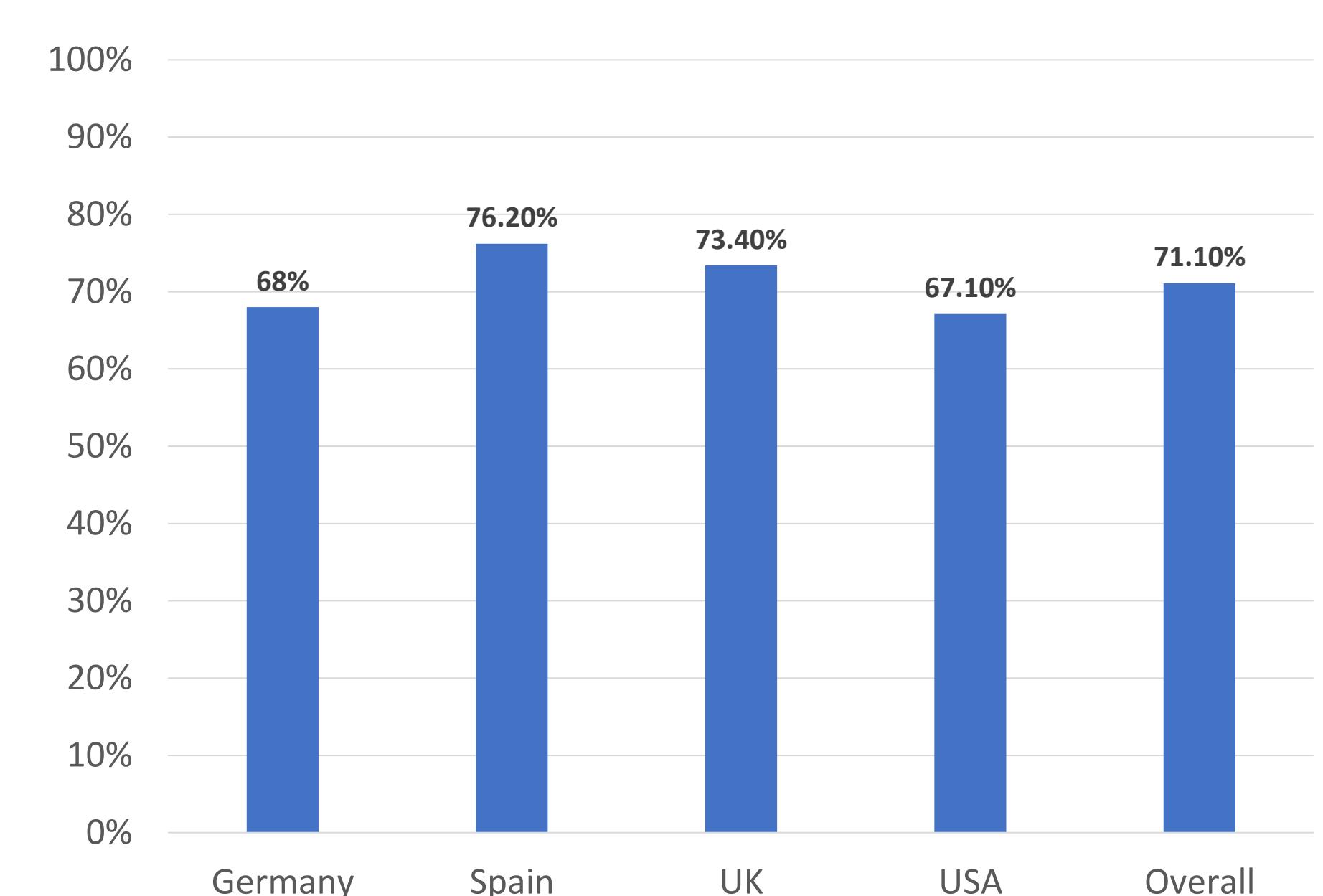


Figure 2. Percentage of respondents reporting severe impact of RD on patient QoL

### Rare disease impact on preventing spending time with family and friends

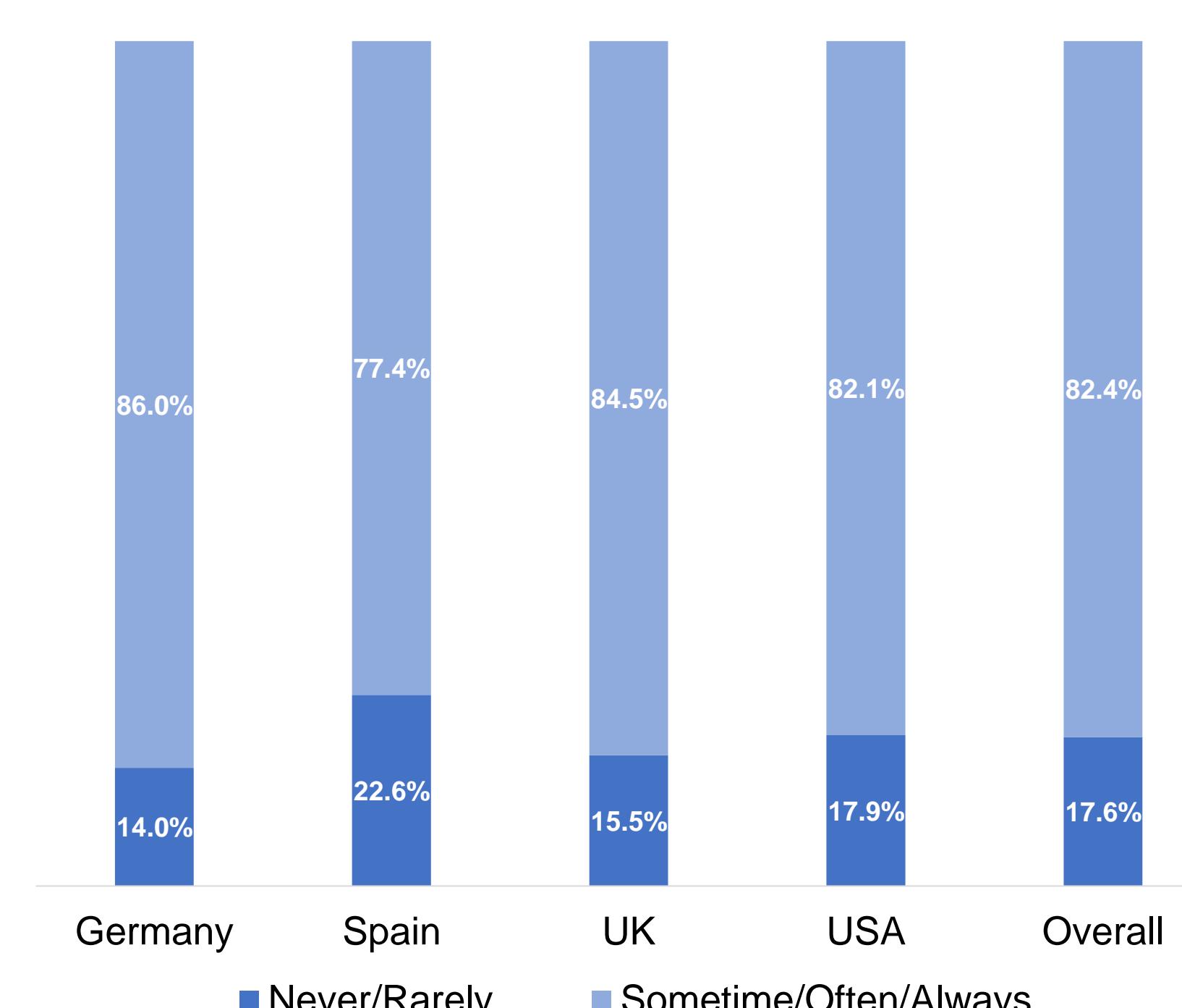


Figure 3. Percentage of respondents reporting impact of RD on time spent with family and friends

### Rare disease impact on exclusion from social events

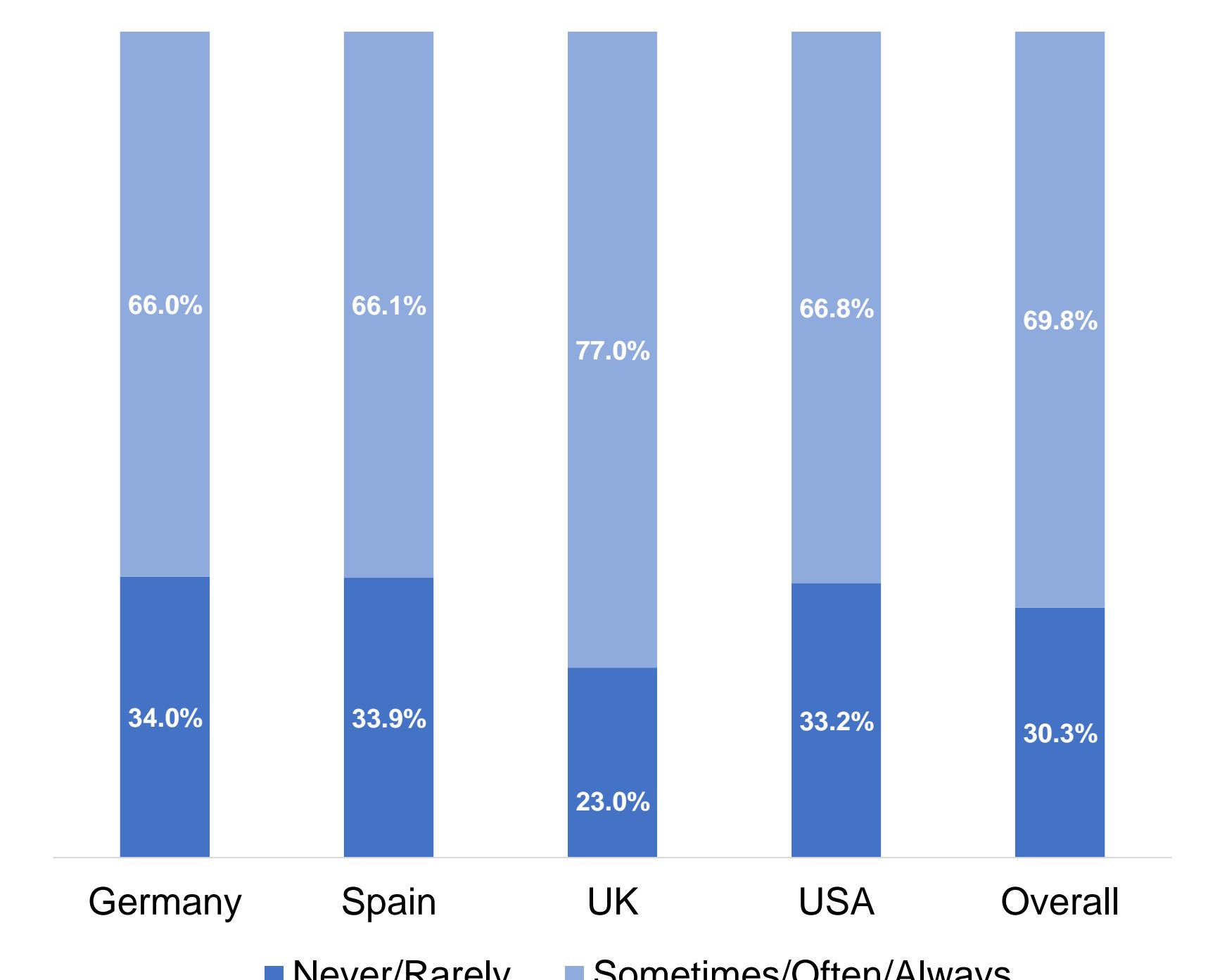


Figure 4. Percentage of respondents reporting impact of RD on exclusion from social events

### Patients feel like a burden to family

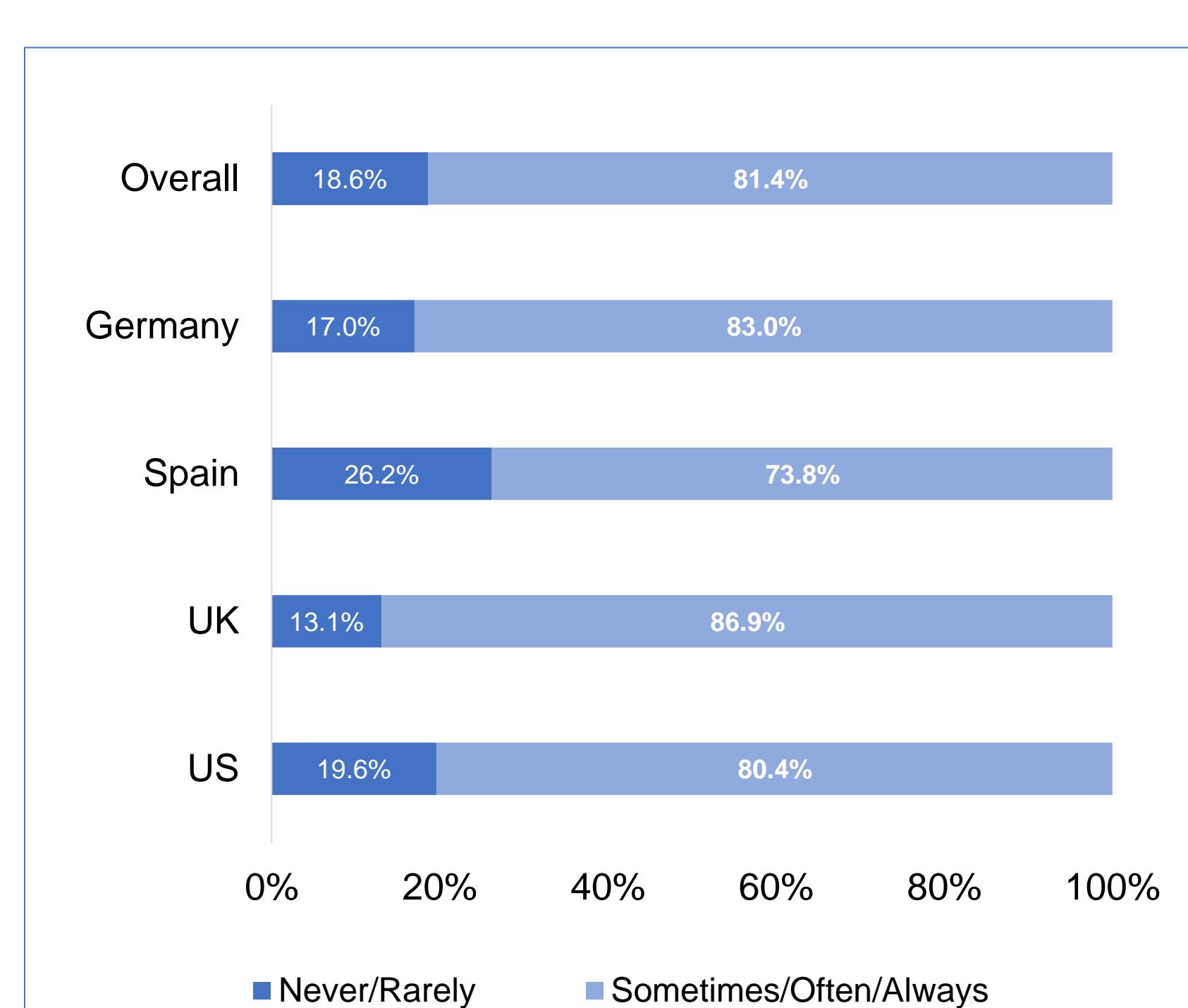


Figure 5. Percentage of respondents reporting impact of RD on time spent with family and friends

### Rare disease impact on relationships

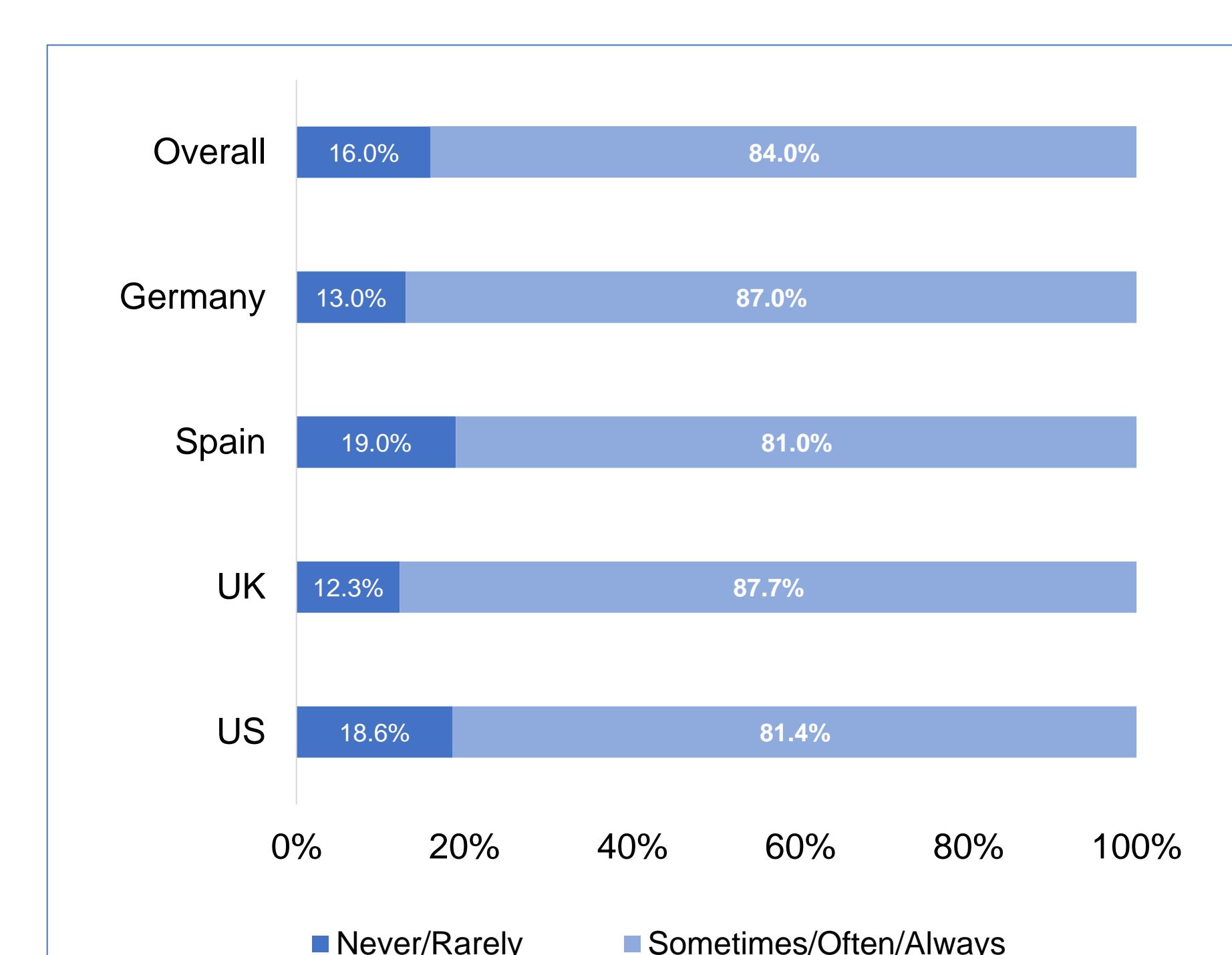


Figure 6. Percentage of respondents reporting impact of RD on exclusion from social events

For more information, contact: [snarayanan@avant-health.com](mailto:snarayanan@avant-health.com)