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Rare Disease Patient and Caregiver Perspectives on Opportunities to Try New Rare Disease Treatments or Participate in Rare Disease Research: Results From An International Survey

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Background

Clinical, humanistic and economic burden of rare diseases (RD) is high, and majority of RDs currently lack treatments to manage the disease burden.

High economic impact of RDs is often driven by the cost of care. Evidence indicates that costs are higher in a scenario without treatment when compared to a scenario with treatment.

More research, new diagnostic tools, or treatments aren't widely available or being developed on a larger scale for the affected population.

RD patients and their families experience hurdles accessing the right diagnostic care and treatment.

Due to small patient populations and limited treatment options, medical professionals except for a few specialists aren't well vested in RD. This could prevent them from assisting patients or provide opportunities for them to participate in research studies or try new treatments.

Objective

To understand rare disease (RD) stakeholder perspectives regarding their opportunities to try new RD treatments, or participate in clinical or real-world research related to their RD.

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 (or their family member's) experience living with RD, including opportunities to participate in research, 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

Across the studied countries, RD patients report seldom being given opportunities to try new treatments or participate in research studies, while they also consider it very important to participate in real-world studies. Doctors and pharmaceutical companies still have ample room to improve on RD patient engagement via research initiatives, which could in turn provide RD patients with opportunities to try new treatments as part of research.

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, with a median age of 44 years; 85% were female; 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, 85% of RD patients and caregivers report never/rarely being provided opportunity to try new RD treatments. (Fig 1)
- Furthermore, 85% of participants consider that it is quite a bit or very much important for individuals with RD to participate in real-world studies (including disease or product registries). (Fig 2)
- However, 86% of survey participants reported to have rarely or never been given an opportunity by their doctor to participate in a research study related to the RD of interest. (Fig 3)

Figure 1. How often are patients/caregivers given an opportunity to try new rare disease treatments?

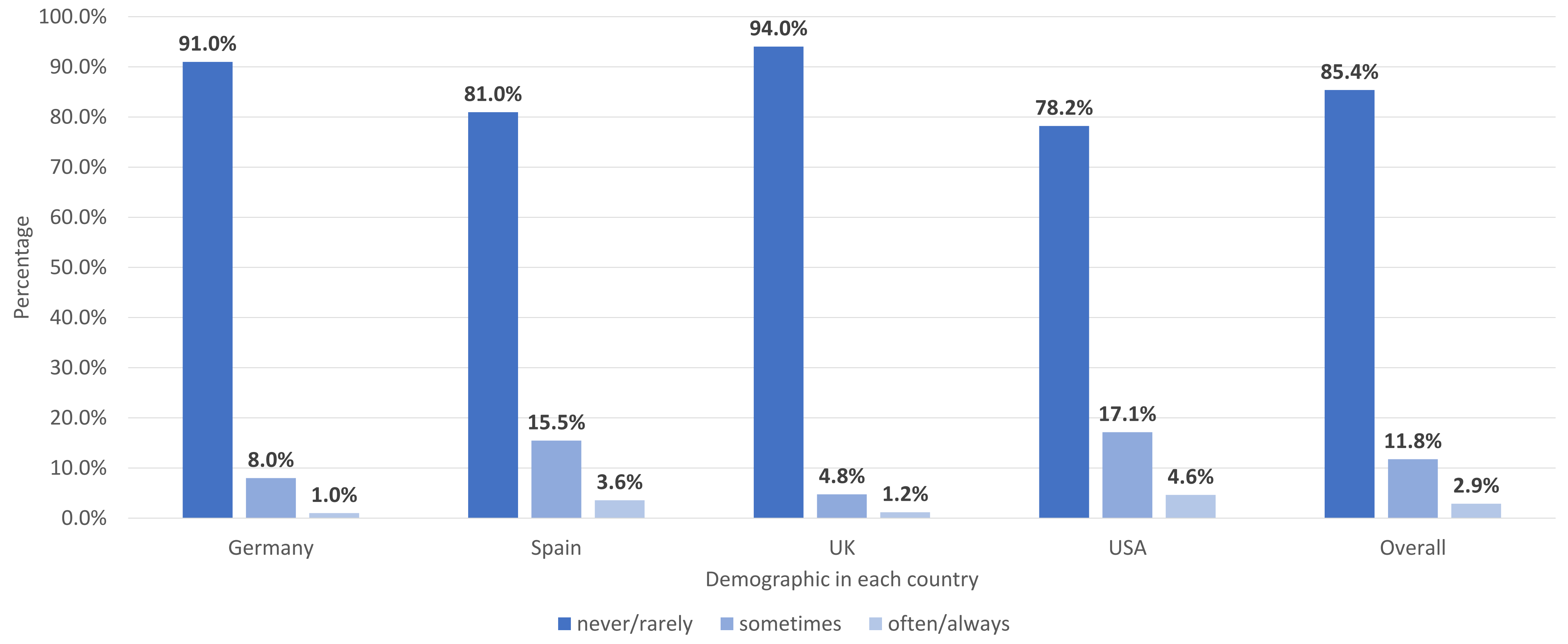


Figure 2. How important is it for individuals with rare disease conditions to participate in real-world studies? (incl. disease or drug registries)

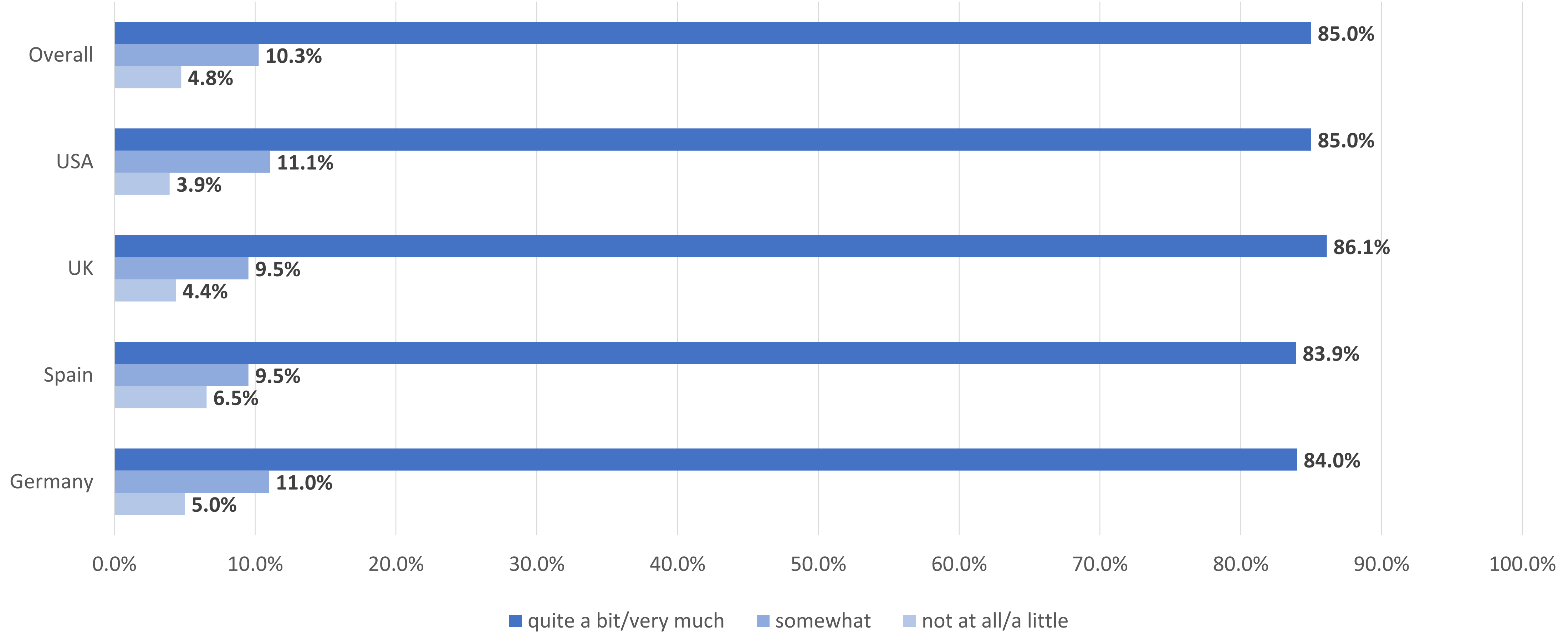
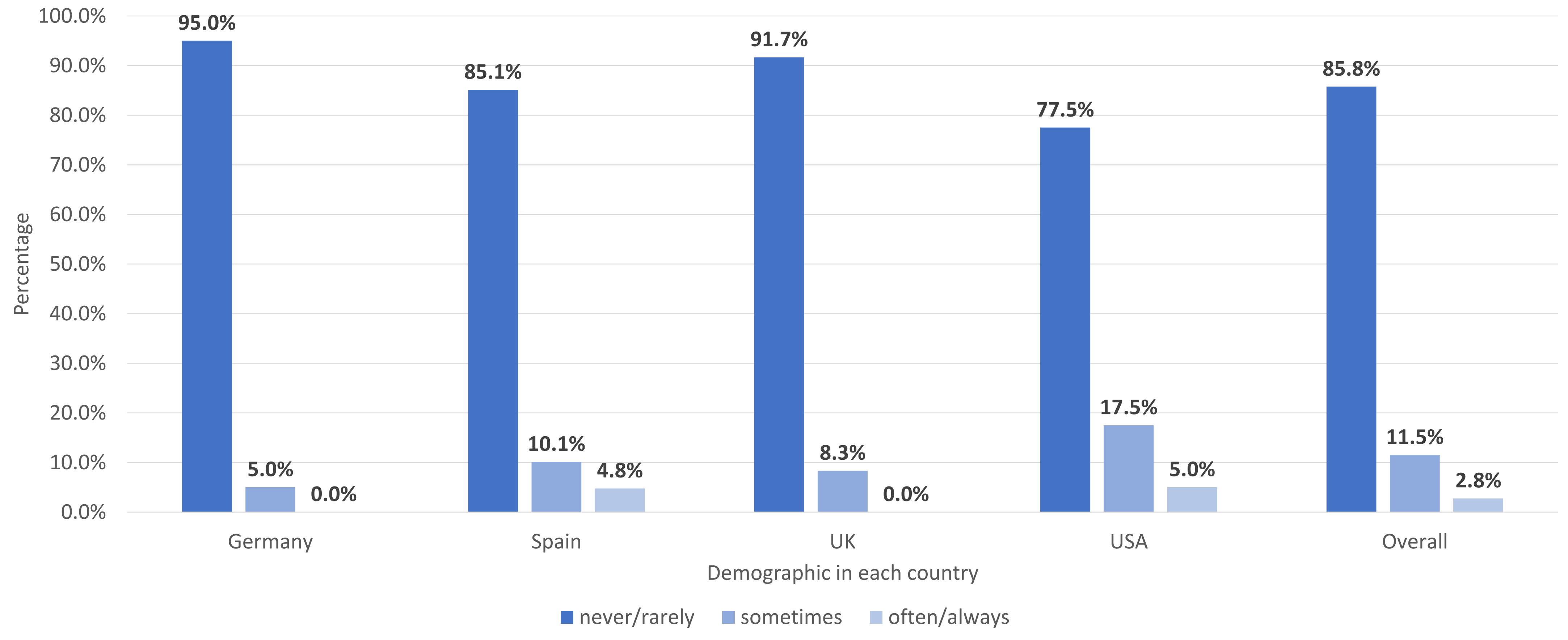


Figure 3. How often patients/caregivers given an opportunity by their doctor to participate in a research study?



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