

Healthcare resource use in Charcot-Marie-Tooth disease in the EU and US: interim results from an international digital real-world evidence study

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Background

Charcot-Marie-Tooth disease (CMT) is a chronic, progressive motor and sensory neuropathy that affects the peripheral nervous system, leading to distal muscle weakness, muscle atrophy and sensory loss.¹

CMT compromises patient lifestyles, everyday activities, and career and family choices.² There is currently no curative treatment, meaning that patients are reliant on supportive care.

CMT is rare, and - until recently - there has been little research into its impact on patients' lives. The collection of real-world data, directly from patients, may therefore provide both new and additional valuable insights.

Objectives

The objective of this analysis was to examine patient-reported healthcare resource use for CMT in the EU and US real-world setting.

Methods

Adults with CMT were recruited to an ongoing two-year international study exploring the real-world burden of the disorder.

Data were collected via CMT&Me, a 'bring your own device' smartphone app, through which participants were asked questions about demographic, CMT management, and quality of life.

This interim analysis (data cut 27 August 2019, approximately ten months after recruitment began) compared annual healthcare professional visit frequency reported by participants from Germany, Italy, Spain, the UK, and the US.

Results

Key demographics for the 953 respondents to this survey are presented in Table 1.

Table 1: Key demographics

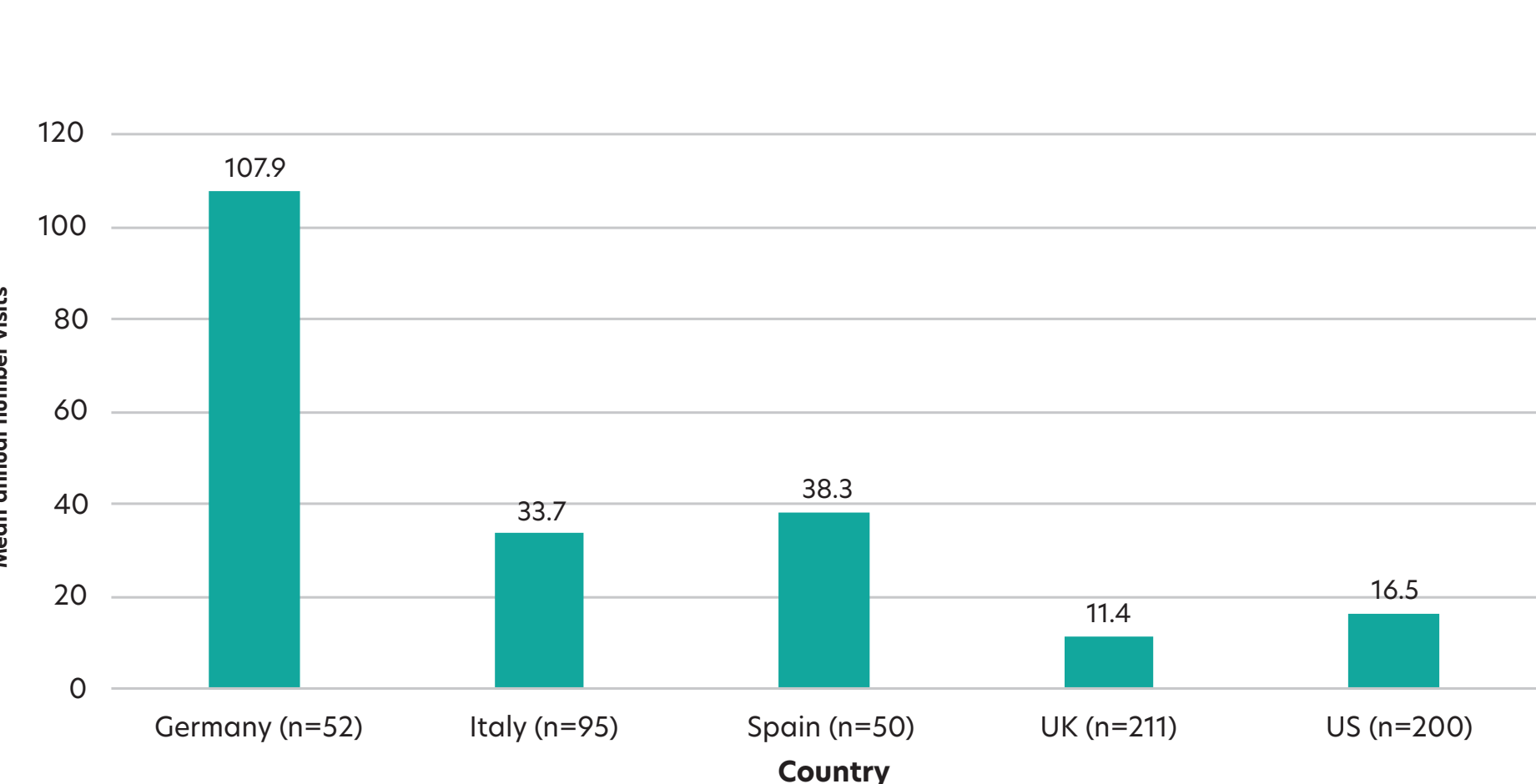
Parameter	Value
Country of residence (n, %)	
Germany (recruitment began January 2019)	74 (7.8)
Italy (recruitment began January 2019)	141 (14.8)
Spain (recruitment began April 2019)	71 (7.5)
UK (recruitment began November 2018)	330 (34.6)
US (recruitment began October 2018)	337 (35.4)
Sex (n, %)	
Male	630 (66.1)
Female	322 (33.8)
Age, years	
Median (range)	47 (18–83)

608 study participants responded to surveys about their annual frequency of CMT-related visits to various members of a multidisciplinary CMT management team.

Figure 1 presents the mean annual number of CMT-related healthcare visits reported by respondents.

The annual number of visits was similar in most countries, apart from in Germany, where a far higher number of visits was reported than elsewhere.

Figure 1: Mean annual number of CMT-related healthcare visits – total visits



Figures 2a and 2b present the distribution of annual CMT-related healthcare visits across various healthcare professionals.

The professionals visited most often were physical/physiotherapists, family doctors, and occupational therapists.

Respondents from Germany appeared to have a particularly high frequency of visits to several healthcare professionals. This was most apparent for physical/physiotherapists, but also noticeable for occupational therapists and podiatrists.

Figure 2a: Mean annual number of CMT-related healthcare visits – by professional

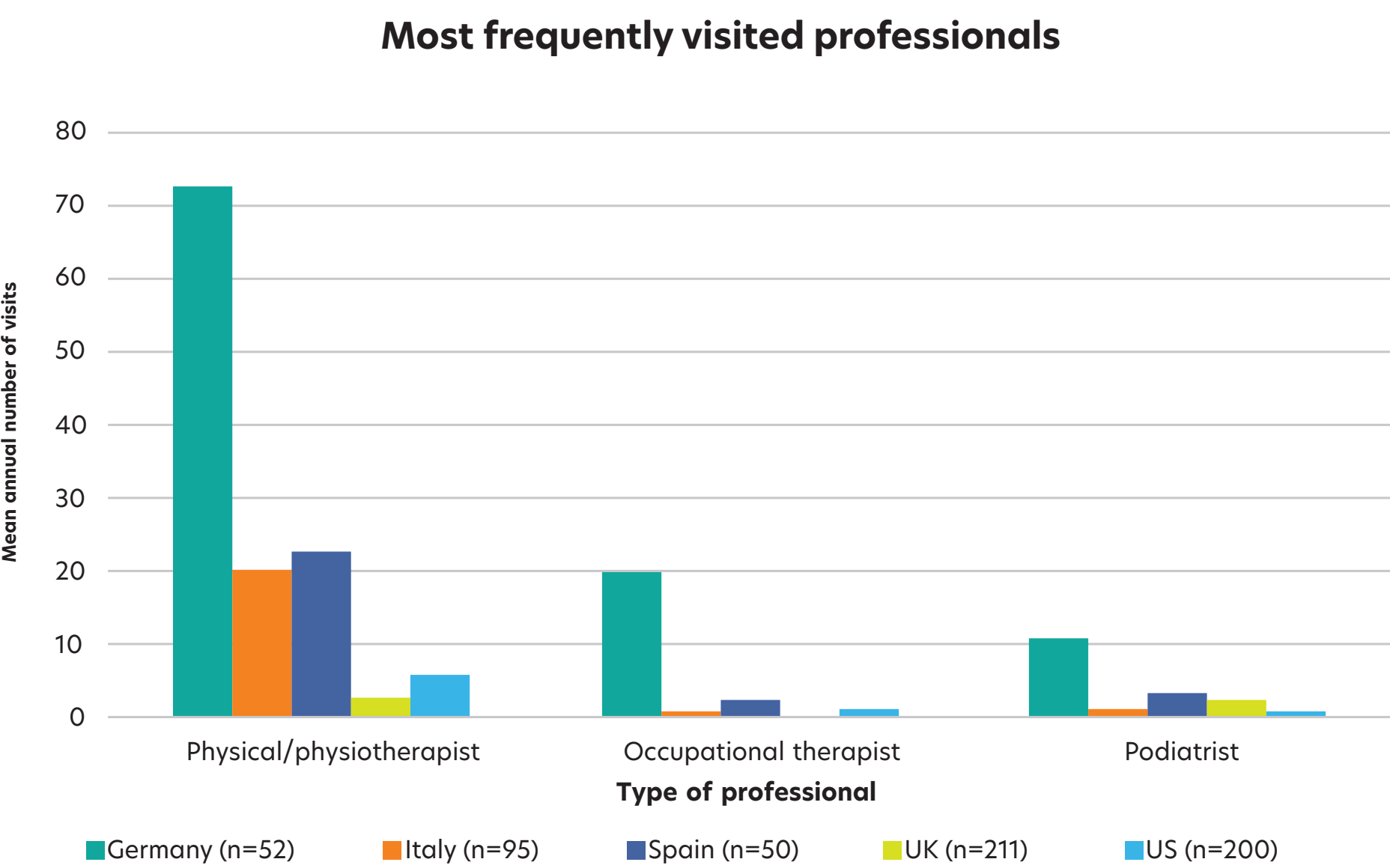


Figure 2b: Mean annual number of CMT-related healthcare visits – by professional

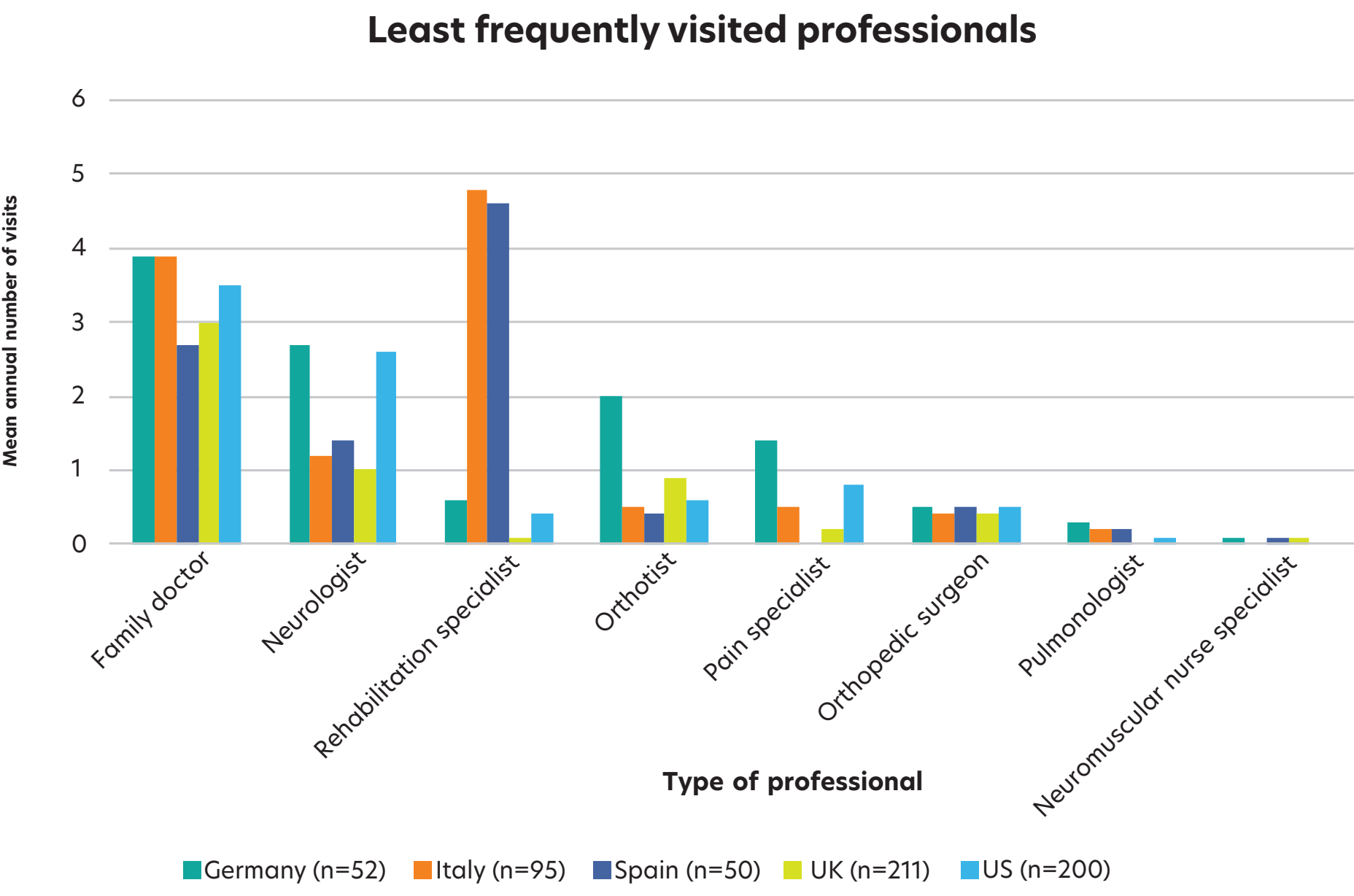


Figure 3 presents the mean annual number of different healthcare professionals visited by respondents.

The number of different professionals visited was similar between countries.

Figure 3: Mean annual number of different healthcare professionals visited for CMT

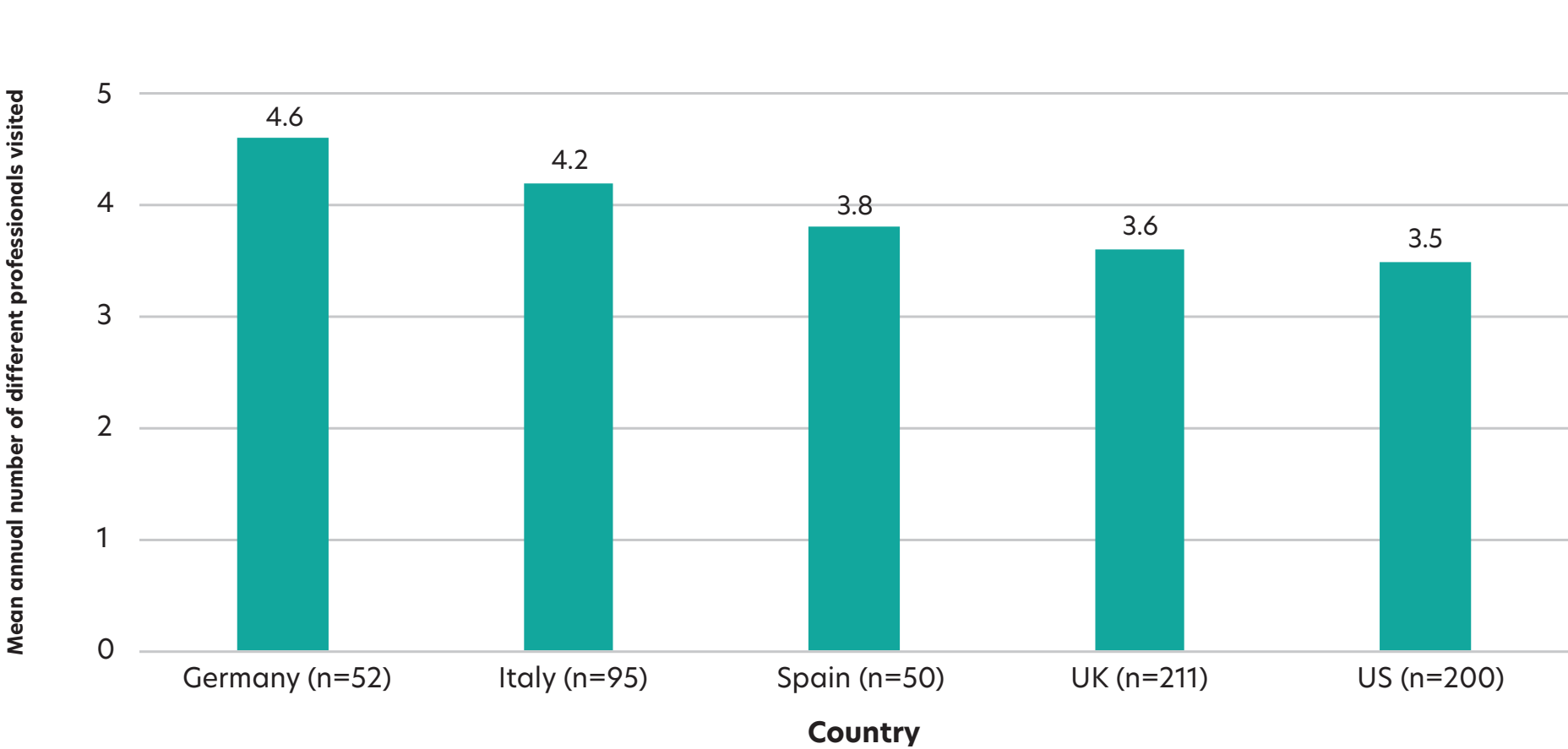
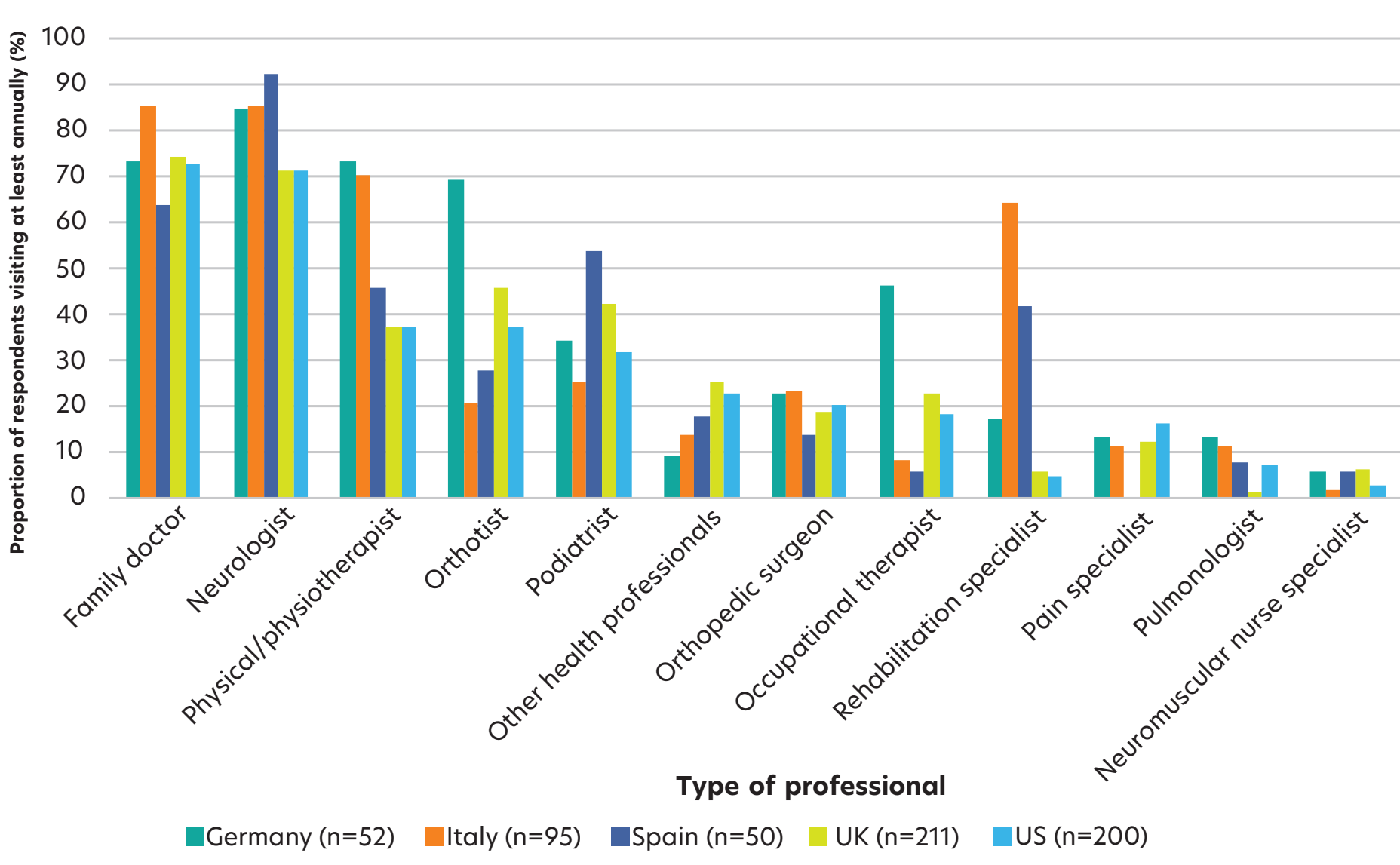


Figure 4 presents the proportions of respondents making CMT-related visits to various healthcare professionals at least once a year.

The professionals visited by the most respondents were family doctors, neurologists, and physical/physiotherapists. Family doctors and neurologists were visited by high proportions of respondents in all countries. However, physical/physiotherapists were visited by many respondents in Germany and Italy, but by fewer respondents in other countries.

Various other professionals were also visited by smaller proportions of respondents from each country. Notably, some of these professionals were visited by high proportions of respondents in particular countries (e.g., occupational therapists in Germany, rehabilitation specialists in Italy and Spain).

Figure 4: Proportion of respondents making CMT-related visits to healthcare professionals annually



Discussion

Healthcare resource use was high among EU and US CMT patients in this digital real-world study.

The annual number of CMT-related healthcare visits was similar in most countries, apart from in Germany, where a far higher number of visits was reported than elsewhere.

Overall, the healthcare professionals visited most frequently were physical/physiotherapists, occupational therapists, and podiatrists. Respondents from Germany appeared to have a particularly high frequency of visits to several healthcare professionals. This was most apparent for physical/physiotherapists, but also noticeable for occupational therapists and podiatrists.

The number of different professionals visited was similar between countries.

The professionals visited by the highest proportions of respondents were family doctors, neurologists, and physical/physiotherapists. Various other professionals were also seen by smaller proportions of respondents from each country.

Taken as a whole, these results indicate that healthcare resource use among people with CMT is high, with patients visiting a range of different healthcare professionals each year. Resource use generally appears to be slightly higher in Germany than in other EU countries and the US. This may warrant further investigation to understand whether there are important differences in the way CMT management is approached in Germany relative to other countries.

Conclusions

This interim analysis showed that the management of CMT involves considerable healthcare resource use.

Participants in this real-world study made CMT-related visits to several different healthcare professionals each year. The frequency and type of visits varied across countries.

The study is ongoing and data will be collected for at least an additional year. Further analysis of a more mature dataset will help better understand the resource use associated with CMT in different countries.

References

¹ Reilly & Shy. J Neurol Neurosurg Psychiatry. 2009; 80(12): 1304-14
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