

Unmet needs of adults with XLH: results from a survey of European patient organizations

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SUMMARY

- X-linked hypophosphataemia (XLH) is a chronic, progressive, musculoskeletal disease associated with lifelong debilitating symptoms; however, little has been published about the burden of disease during adulthood. This survey aimed to identify and prioritize the unmet needs of adults with XLH in Europe.
- Representatives of patient organizations in 11 countries provided responses to a questionnaire. The results indicate extensive unmet needs across Europe, even regarding recognizing the need for treatment in adulthood.
- Overall, the findings highlight disparities across Europe in the care and support provided to adults with XLH and warrant further investigation.

INTRODUCTION

- X-linked hypophosphataemia (XLH) is a chronic, progressive, musculoskeletal disease (Skrinar *et al.* 2019).
- XLH has historically been considered a childhood disease; however, it is now understood that adults with XLH experience a spectrum of debilitating symptoms and disease sequelae that reduce quality of life and represent a considerable burden to the individual, society and the healthcare system (Chesher *et al.* 2018; Skrinar *et al.* 2019; XLH Network, Inc. 2019).
- This survey aimed to identify and prioritize the unmet needs of adults living with XLH in Europe.

METHODS

- Twelve national patient organizations in 11 countries (**Table 1**) were surveyed in August and September 2019; in Italy, two groups provided a joint response.
- Patient group leaders were asked to respond to the following instructions with responses that would represent the views of their organization's members.
 - Rate the level of need for improvements (as high, moderate, low, already satisfied or not applicable) in 15 areas relating to XLH in adulthood.
 - These included aspects of healthcare, disease awareness, social care and financial support, which were identified from a systematic literature search and a wider review of internet-based sources.
 - Identify the top five priorities for each country from among these 15 areas.
 - Add additional areas of unmet need, as appropriate.

RESULTS

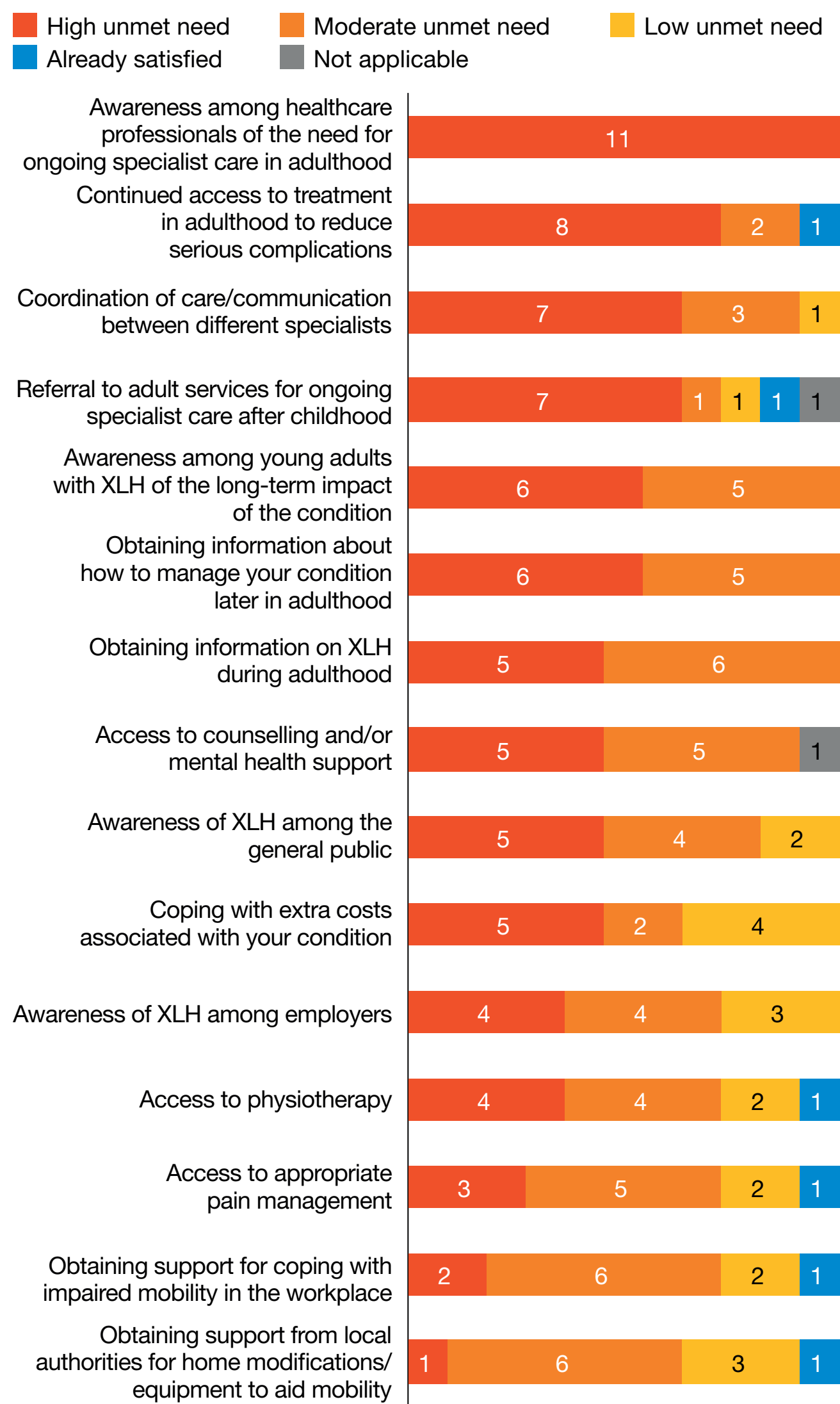
- All countries reported a high need for better 'awareness among healthcare professionals of the need for ongoing specialist care in adulthood' (**Figure 1**).
- The unmet needs that were most frequently included in the top five priorities of different countries are shown in **Figure 2**.
 - The unmet needs rated as most important by the patient groups in each country were: referral to adult services (France, Italy, Portugal, Spain and Sweden); awareness among healthcare professionals of the need for ongoing specialist care (Finland, Germany and the UK); coordination of care (Austria and Denmark); and access to physiotherapy (Belgium).
- Analysis of the responses to individual aspects of XLH in adulthood identified differences across Europe in unmet needs.
 - For example, better access to physiotherapy and better access to appropriate pain management were rated as 'high' or 'moderate' needs by most countries but as 'already satisfied' by the Portuguese patient society (**Figures 3 and 4**).
 - Unmet needs for obtaining better support to aid mobility at home and at work were rated as 'high' or 'moderate' by most countries but as 'already satisfied' by the German patient society (**Figures 5 and 6**).
- Additional unmet needs highlighted by the patient groups included access to specialist dental care (Austria, Sweden and the UK) and access to information about the disability rights of adults with XLH (Finland, Portugal and Spain).

Table 1. National patient organizations that participated in the survey.

| Patient group | Country |
|-------------------------------|--------------------|
| Phosphatdiabetes Österreich | Austria |
| XLH Belgium | Belgium |
| XLH Patientforeningen | Denmark |
| Kalfos ry | Finland |
| RVRH-XLH | France |
| Phosphatdiabetes e.V. | Germany |
| AISMME | Italy ^a |
| AIFOSF | Italy ^a |
| ANDO | Portugal |
| AERyOH | Spain |
| XLH Svenska Patientföreningen | Sweden |
| XLH UK | UK |

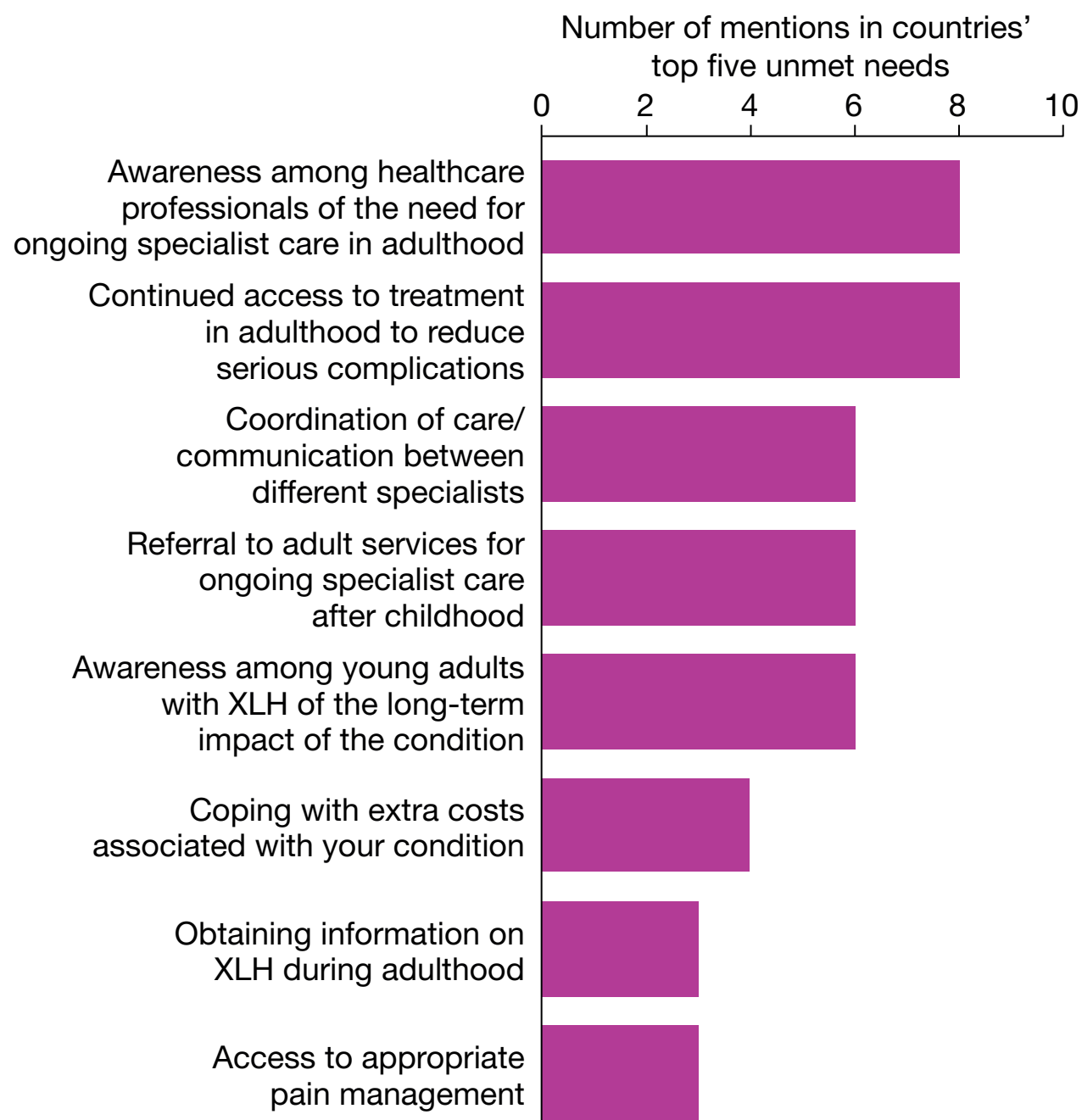
^aThe two Italian groups provided a joint response to the survey. XLH, X-linked hypophosphataemia.

Figure 1. Level of need for improvement in areas relating to XLH in adulthood rated by patient groups from across Europe.



Data shown are number of countries that selected each rating. XLH, X-linked hypophosphataemia.

Figure 2. Unmet needs selected by national patient groups as part of their top five priorities.



Shows all needs selected by at least three countries. XLH, X-linked hypophosphataemia.

CONCLUSIONS

- Overall, the findings indicate that adults with XLH in Europe have substantial unmet needs.
- Disparities between countries in how the needs of adults with XLH are met indicate a lack of defined care for these individuals.
- Despite differences between national patient organizations in their prioritization of the needs, greater awareness among healthcare professionals and patients of the need for continued care in adulthood is required in all countries surveyed.
- Exploration of the impact of differences across Europe in the care provided to adults with XLH is warranted.

REFERENCES

- Chesher D *et al.* *J Inher Metab Dis* 2018;41:865–76.
- Skrinar A *et al.* *J Endocr Soc* 2019;3:1321–34.
- XLH Network, Inc. Voice of the patient report, 2019: https://xlhnetwork.org/application/files/9415/7323/0655/VOP_and_Transcripts.pdf (Accessed 24 March 2020).

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DISCLOSURES

PH is a member of RVRH-XLH (a member of the International XLH Alliance), which has received funding from Kyowa Kirin for the organization of educational events, and has provided unpaid consulting services to Kyowa Kirin. PH is employed by GSK. This poster was prepared by PH in his personal capacity. LS has received honoraria from Kyowa Kirin for lectures and advice. LP is employed by Kyowa Kirin.

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Figure 3. Need for better access to physiotherapy.

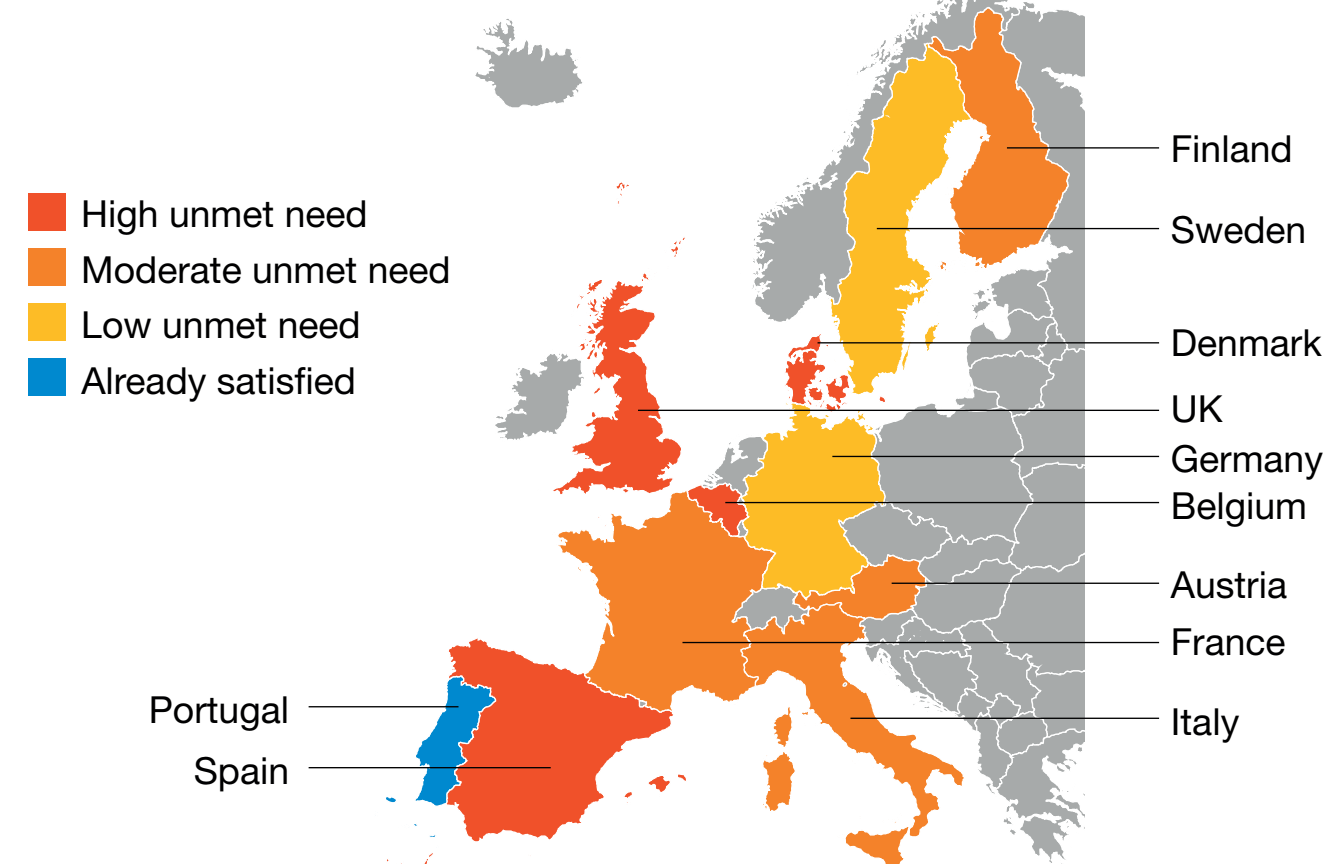


Figure 4. Need for better access to appropriate pain management.

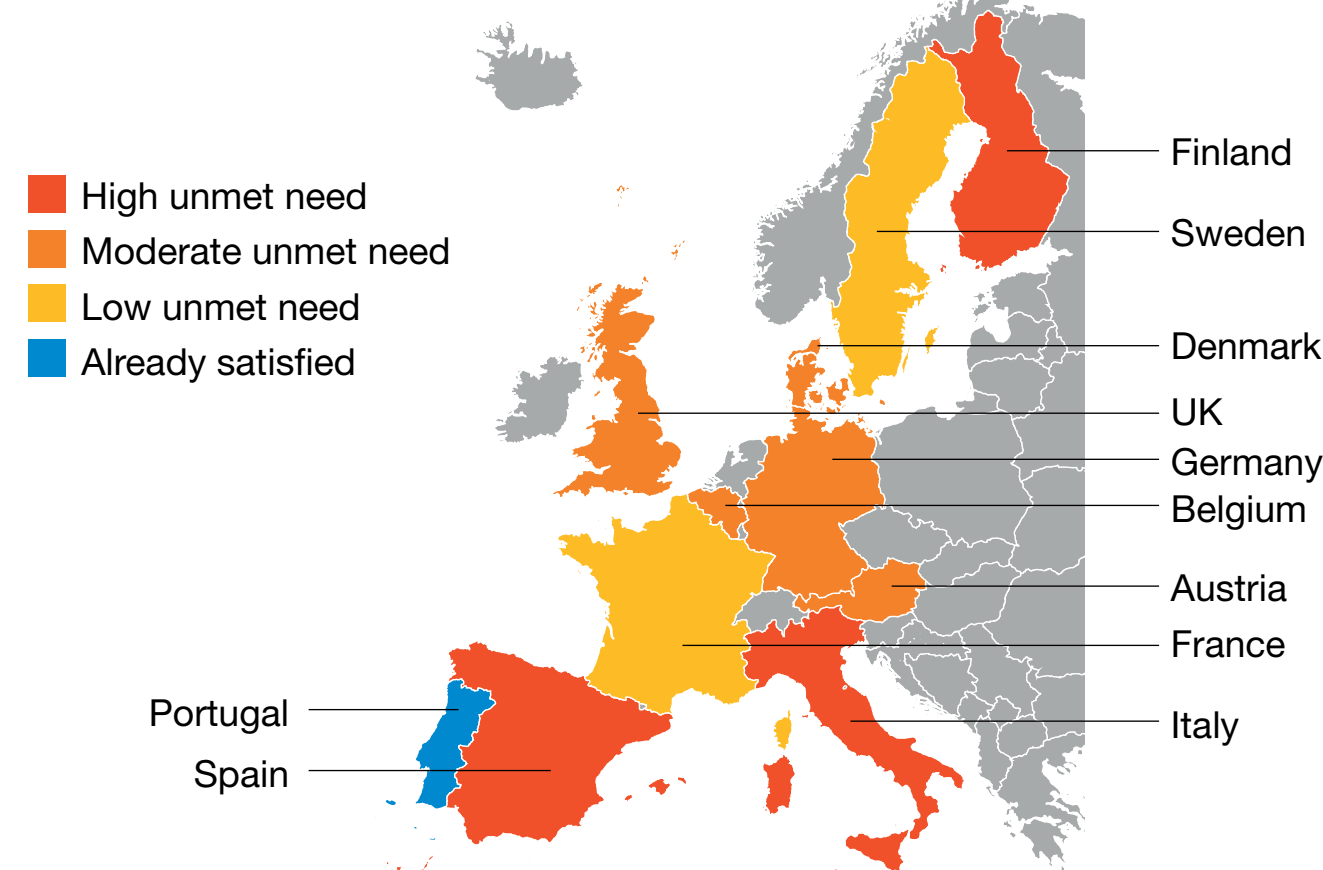


Figure 5. Need for better support from local authorities to aid mobility at home.

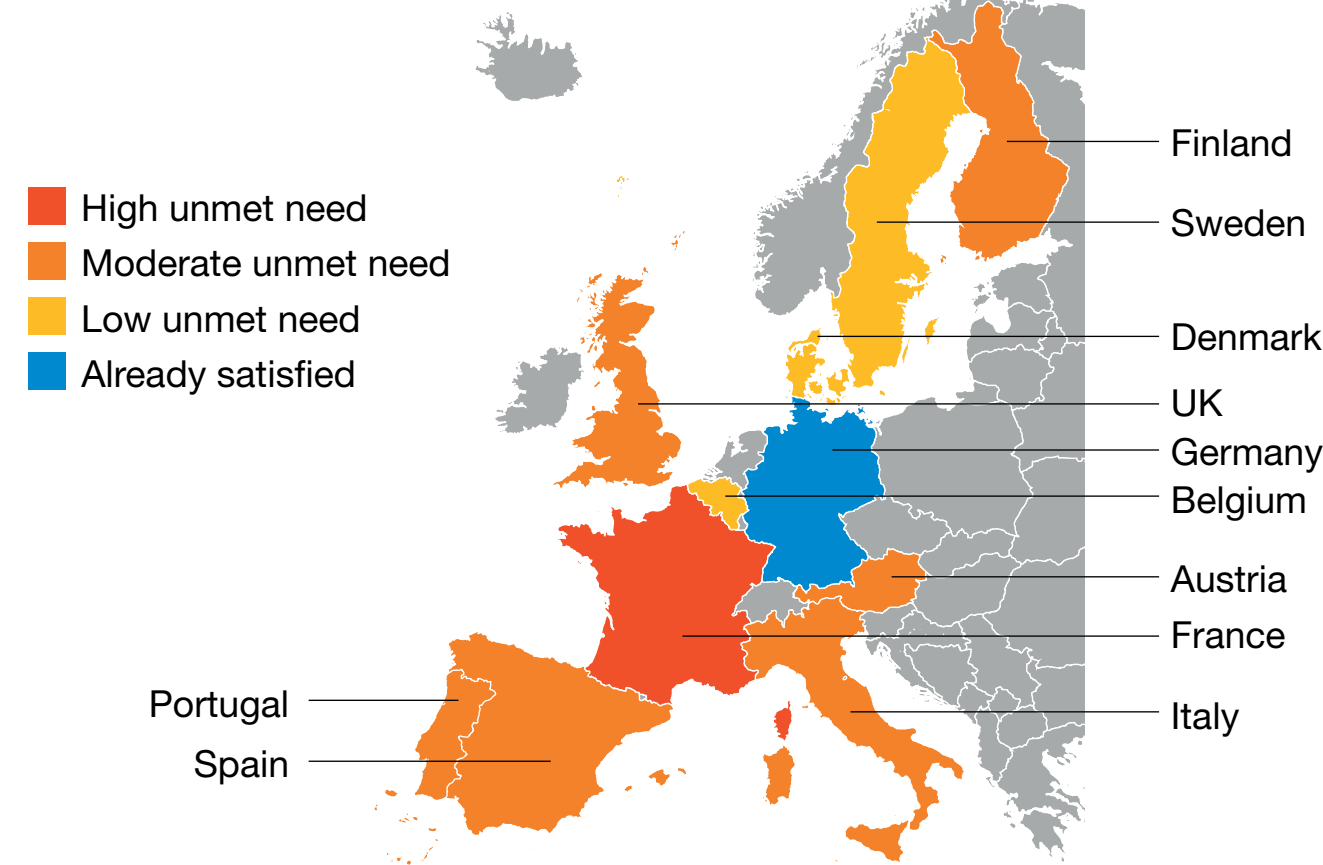


Figure 6. Need for better support to aid mobility in the workplace.

