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

Pol Harvenst,1 Lothar Seefried,2 Lindsay Perera3
1FVRH-XLH, French association of patients with XLH, Suresnes, France; 2Orthopedic Institute, König-Ludwig Haus, Würzburg, Germany; 3International Advocacy Manager, Kyowa Kirin International, Galashiels, UK

SUMMARY
- X-linked hypophosphataemia (XLH) is a chronic, progressive, musculoskeletal disease associated with 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 (Skirinar 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 (Cheisher et al. 2018; Skirinar 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:
  1. Rate the level of need for improvements (as high, moderate, low, already satisfied or not applicable) in 15 areas relating to XLH in adulthood.
  2. Identify the top five priorities for each country from among these 15 areas.
  3. Add additional areas of unmet need, if 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.
- Unmet needs relating to access to physiotherapy and access to appropriate pain management were rated as ‘high’ or ‘moderate’ needs by most countries but as ‘already satisfied’ by the German patient society (Figures 3 and 4).
- 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).

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 needs.
- 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

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

Patient group | Country
---|---
Phosphosylates Österreich | Austria
XLH Belgium | Belgium
XLH Patiëntenverenigingen | Denmark
Kalfag | Finland
RiFH-XLH | France
Phosphosylates e.V. | Germany
AEHAE | Italy
AAPF | Spain
AEP | Portugal
AEF/Oh | Spain
XLH Svenska Patiëntenverenigingen | Sweden
XLH UK | UK

This is a rare disease project as part of the survey. XLH is an inherited hypophosphataemia.

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