

Patients' view on the unmet need in endocrine medical research

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Theme 4: When therapies meet the needs: enabling a patient-centric approach to therapeutic development

INTRODUCTION

- The mission of Endo-ERN is to reduce inequalities in care for patients with rare endocrine conditions
- Patient representatives are involved in steering committees
- Work package 'Research & Science' aims to capture unmet needs of patients

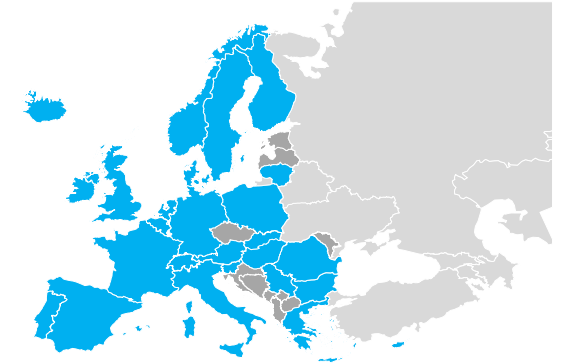
METHODS

- Survey in 22 languages on unmet needs in research
- Distributed via patient advocacy groups, reference centres of Endo-ERN, and Orphanet newsletter
- Patients scored priorities on a 3 point scale (1, most; 3, least important)
- Results were analyzed comparing gender and disease groups (MTGs)

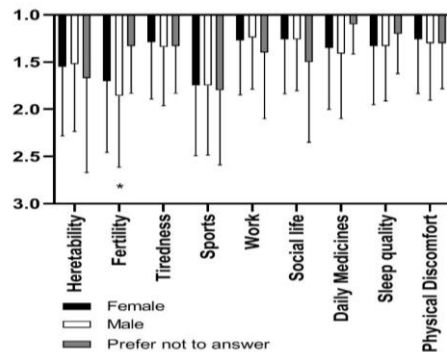
RESULTS

- 1387 individual responses from 27 European countries
- Eastern Europe underrepresented
- Most interest in research concerning ability to work and participate in social life and tiredness
- Interest in research on tiredness differed between MTGs

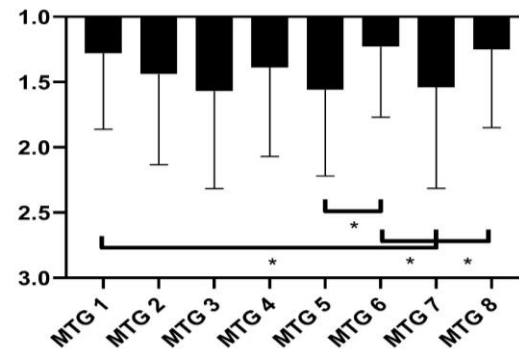
COUNTRIES WITH RESPONDENTS



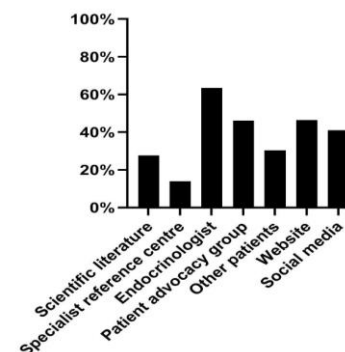
PRIORITIES BY GENDER



TIREDNESS BY MTG



SOURCES OF INFORMATION



CONCLUSIONS

- We report on a large sample of European patients with a rare endocrine disease
- Patients with rare endocrine diseases experience unmet needs in research
- These needs differ between disease groups

FUTURE

- Answers to open questions will be analyzed for more detailed assessment of patients' needs
- Discussion on results within MTGs