11EME JOURNÉE ANNUELLE FIRENDO

MERCREDI 4 DÉCEMBRE 2024

PARIS 14e

FIRENDO

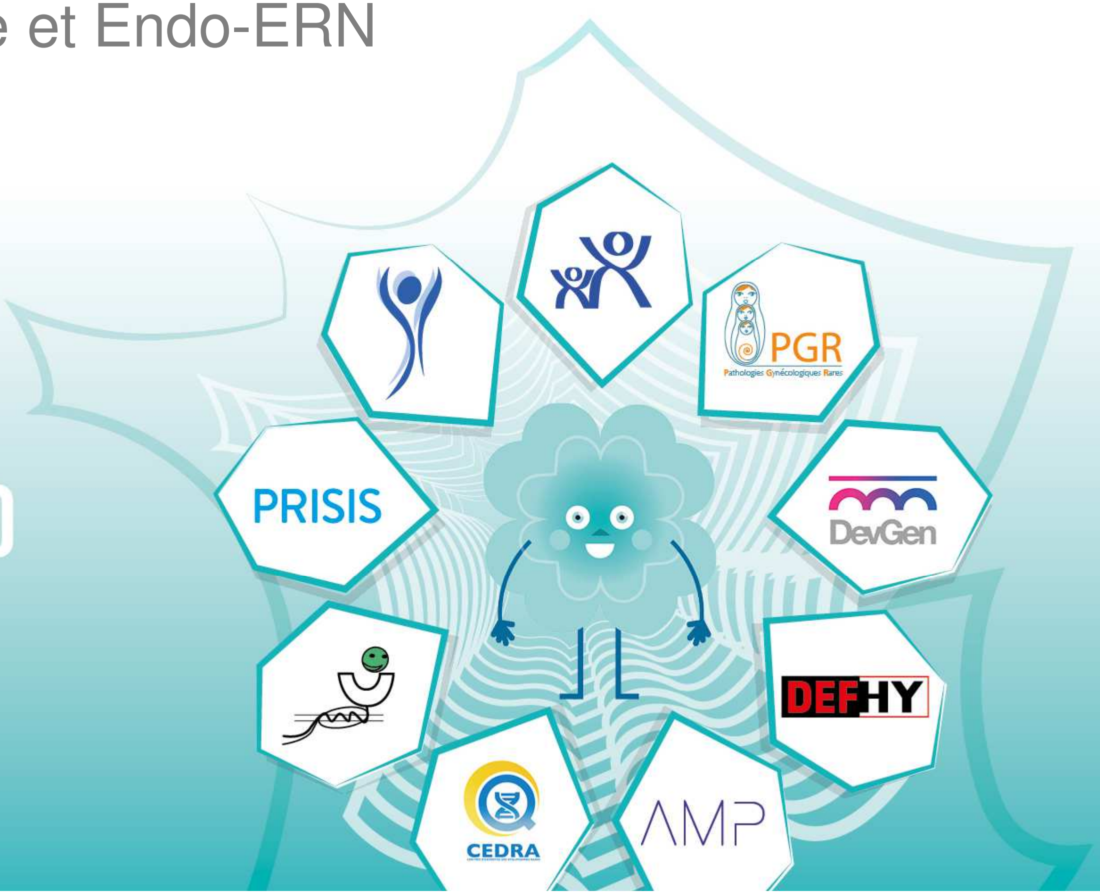
FILIÈRE MALADIES RARES ENDOCRINIENNES

Overview on ePAG activities in ENDO-ERN

Johan P de Graaf
Fondation Hypophysaire Néerlandaise et Endo-ERN

11EME JOURNÉE ANNUELLE FIRENDO
MERCREDI 4 DÉCEMBRE 2024 PARIS 14e

FIRENDO
FILIÈRE MALADIES RARES ENDOCRINIENNES



Subjects to be covered today

- European Reference Networks and Endo-ERN
- European Patient Advocacy Groups, ePAGs
- ePAGs within Endo-ERN; are they happy?
- Zooming in on ePAG activities within Endo-ERN
- Measuring of patient engagement within Endo-ERN
- Being well-prepared as a patient representative on the European stage

European Reference Networks

- 24 ERNs Launched in 2017
- Virtual networks with health care providers, researchers and patients
- Aims to provide high-quality cost-effective health care for rare disease patients
- Cross border health directive (2011/24/EU)
- Online advisory panels (CPMS, clinical patient management system)
- Research initiatives, guideline development, patient information
- Education: webinars, exchange programs
- Unique opportunities for patient representatives

European
Reference
Network

European Reference Network on Rare Endocrine Conditions

- 105 centres of expertise in 28 countries
- Endo-ERN’s mission is to reduce and ultimately abolish inequalities in care for patients with rare endocrine conditions in Europe through facilitating knowledge sharing and related healthcare and research. Endo-ERN provides equality between paediatric and adult patients.
- Cooperation on research, education, quality of care, guidelines and the provision of information
- Paediatric, Adult endocrinology and patient representation incorporated in the governance structure
- **Endo-ERNs motto: Expert multi-disciplinary care throughout life**

Endo-ERN

What are the European Patient Advocacy Groups?

- Commonly referred to as **ePAGs**: the patients who are involved in the ERNs
- The definition of Eurordis:
- *European Patient Advocacy Groups (ePAGs) are patient groups that bring together rare disease patient advocates who are actively involved in the 24 European Reference Networks, working in partnership with clinicians and researchers. Each of the 24 Networks focuses on a particular disease area and has its own ePAG*
- Currently within Endo-ERN 19 patient representatives are involved from 9 different countries

European
Patient
Advocacy
Group

ePAGs represent the interests and needs of the patient community

- to ensure that the needs of people living with a rare disease drive the activities of each European Reference Network
- to champion the diversity of views of the wider patient community relevant for each ERN, and not just of their own disease area
- to work with their national and European networks in order to facilitate two-way communication between the European Reference Networks and the patient community
- to work in partnership with the clinicians to support the development of clinical practice guidelines and other clinical decision support tools and contribute to research, education, information and awareness activities
- The position of an ePAG advocate is a voluntary position and does not involve any financial compensation. Individuals must be endorsed by a patient organisation based in a European country to join as an ePAG advocate and be involved in ERN working groups

The map of Endo-ERN

- Endo-ERN is represented in all EU member states plus Norway, making it the largest ERN
- 19 ePAG representatives from 9 different countries
- Under representation of Eastern Europe
    - Language difficulties
    - Lacking infrastructure of patient organisations
- Current representation for France in Endo-ERN:
    - Association Grandir (Nathalie Ferard)
    - Vivre sans Thyroide (Beate Bartés)
    - Association du syndrome de Wolfram (Nolwen leFloch)

The structure of Endo-ERN

- Main Thematic groups (MTGs)
- Work Packages (WPs)
- Patient involvement in both MTGs and WPs
- Patients are steering commitee members, besides an adult and a paediatric chair

Nienke Biermasz Adult chair
Leiden University Medical Center (Leiden , Netherlands)


Nienke Biermasz

Evangelia Charmandari Paediatric chair
Aghia Sophia Children's Hospital (Athens, Greece)

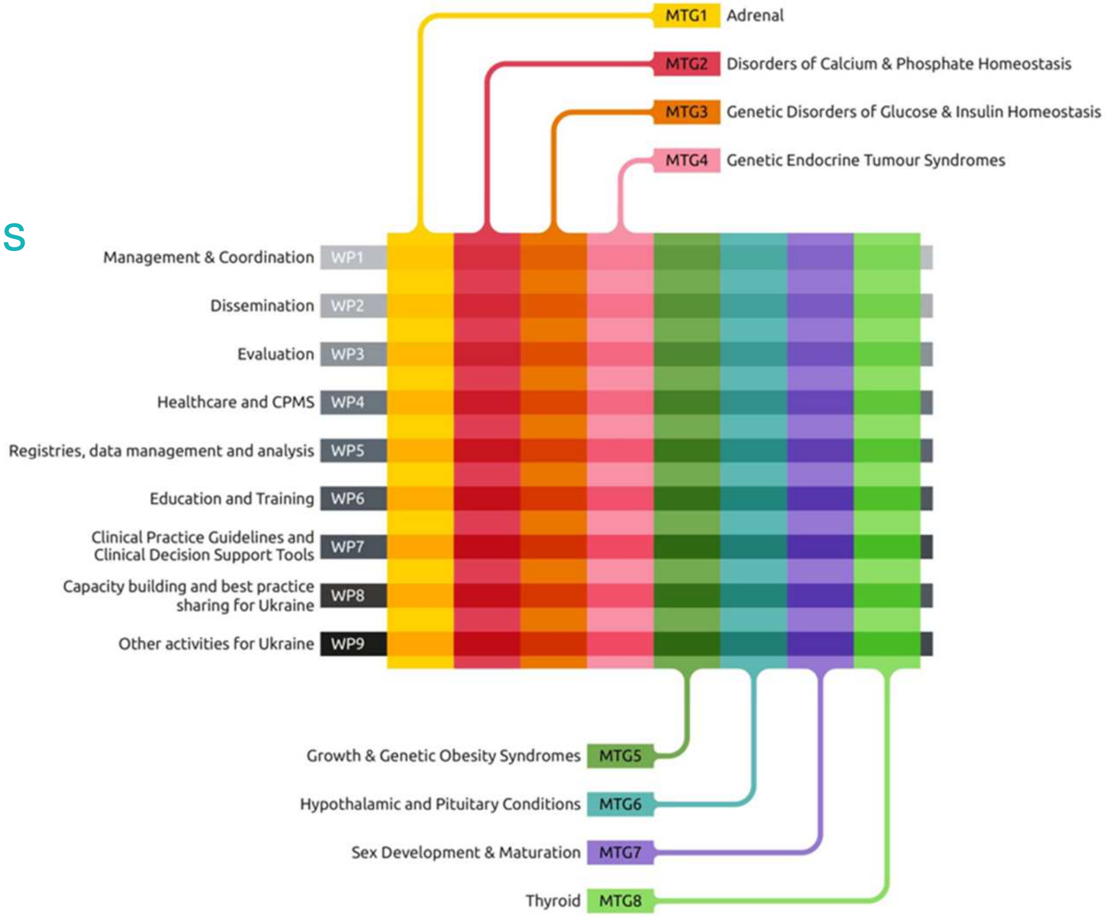

Evangelia Charmandi

Johan de Graaf ePAG representative
Dutch Pituitary Foundation (Nijkerk, Netherlands)


Johan de Graaf

Diana Vitali ePAG representative
SOD ITALIA – Italian organization for septo optic dysplasia and other neuroendocrine disorders (Roma, Italy)


Diana Vitali

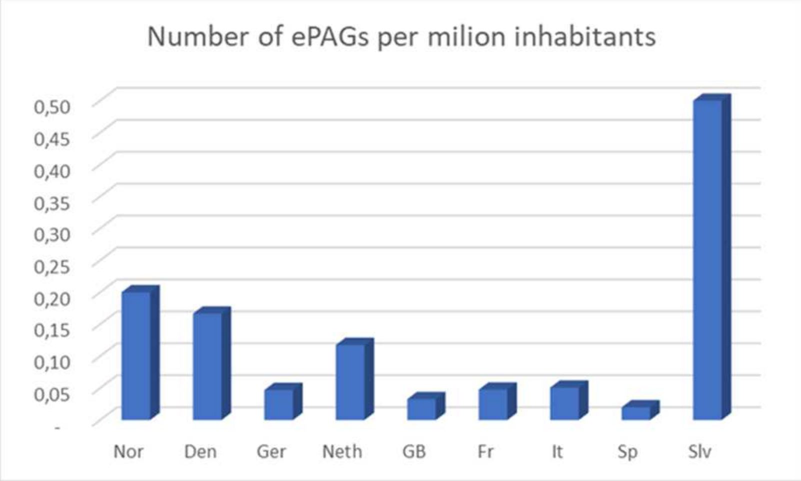

MTG1 Adrenal
MTG2 Disorders of Calcium & Phosphate Homeostasis
MTG3 Genetic Disorders of Glucose & Insulin Homeostasis
MTG4 Genetic Endocrine Tumour Syndromes

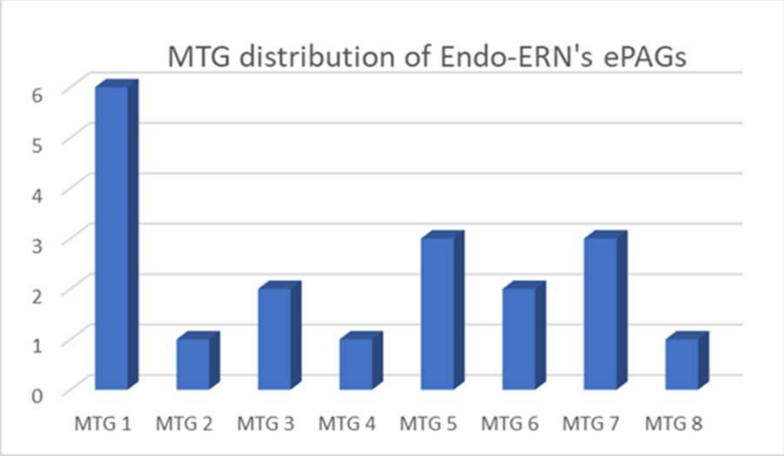
Management & Coordination WP1
Dissemination WP2
Evaluation WP3
Healthcare and CPMS WP4
Registries, data management and analysis WP5
Education and Training WP6
Clinical Practice Guidelines and Clinical Decision Support Tools WP7
Capacity building and best practice sharing for Ukraine WP8
Other activities for Ukraine WP9

Growth & Genetic Obesity Syndromes MTG5
Hypothalamic and Pituitary Conditions MTG6
Sex Development & Maturation MTG7
Thyroid MTG8

Survey (2023) on ePAG involvement within Endo-ERN (I)

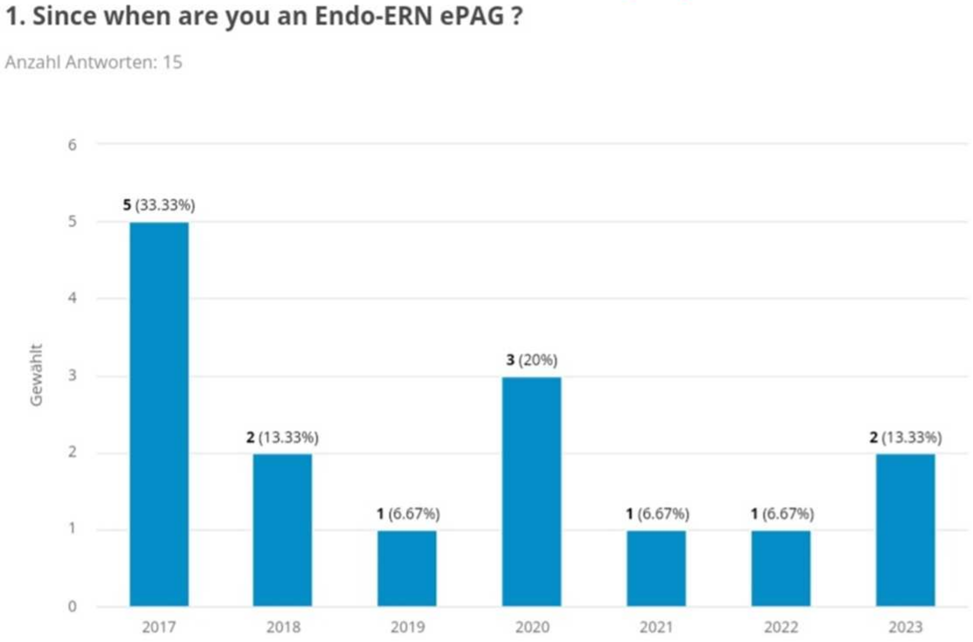
- Country involvement compared to population size gives interesting insights
- Adrenal disorders seems to be popular
- Finding ePAGs for rarer endocrine disorders is challenging, some ePAGs are on their own in a MTG
- Very international group of patient representatives
- Exchange of best practices
- Friendships





Survey (2023) on ePAG involvement within Endo-ERN (II)

- The original group of 2017 is almost entirely still in office
- New blood is added constantly
- Though there are some challenges, patients are satisfied within Endo-ERN
- Over 70% replied to stay within Endo-ERN, rest wasn't sure (and didn't say no!). Mostly because time availability

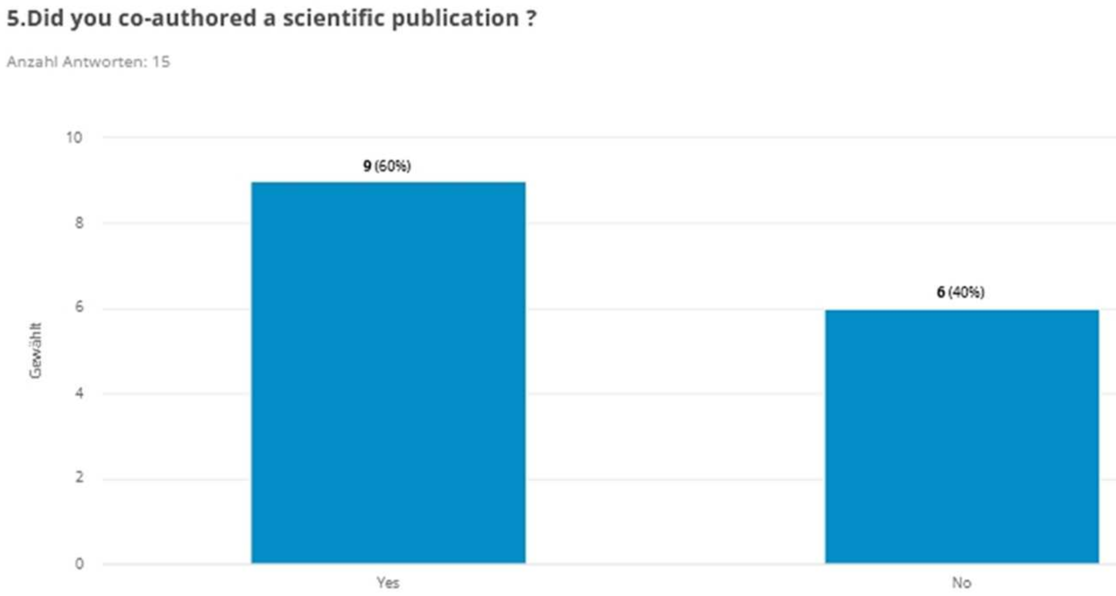


1. Since when are you an Endo-ERN ePAG ?

Anzahl Antworten: 15

Survey (2023) on ePAG involvement within Endo-ERN (III)

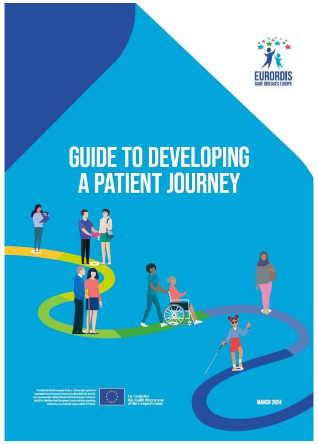
- 60% of the ePAGs co-authored (or even authored) a scientific publication
  - 25% co-authored 1 publication
  - 12,5% co-authored 3 publications
  - 50% co-authored 4 publications
  - 12,5% co-authored 6 publications
- almost 90% of the ePAGs want to be involved in one of more scientific publications in future!


5.Did you co-authored a scientific publication ?

Anzahl Antworten: 15

Zooming in on patient activities: What are patients doing?

- Involvement in the development of Clinical guidelines
- Development of Patient Journeys
- Attending international conferences
- Providing the patient perspective in webinars
- Involvement in research projects
- Performing research by means of surveys
- Playing a role in the Endo-ERN registry project
- → Measuring patient involvement within Endo-ERN: Notebook



www.eurordis.org

International Conferences

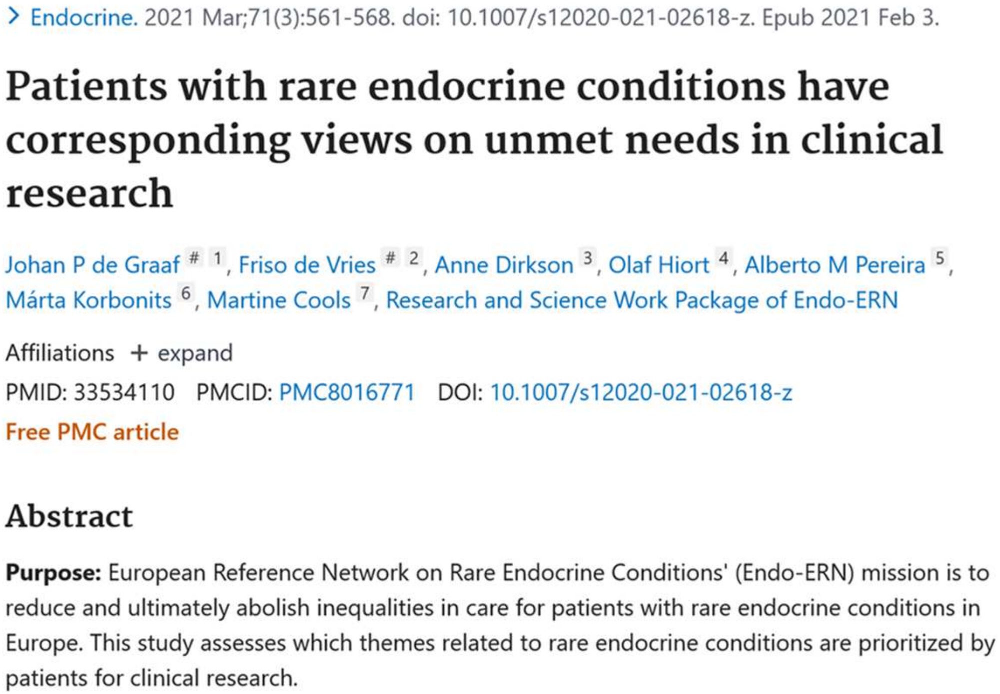
- Being able to attend congresses of ESE and ESPE
- From spectator to participant
- ESE PAG Affiliate Membership Board
  - Established 2022 under the ESE presidency of professor Jerôme Bertherat
  - Representation of ESE and delegates of PAG members
  - Discussion forum between ESE and PAG Members
  - Prioritising on:
    - Patient focussed educational initiatives
    - Building patient advocacy and support across Europe
    - Inequalities in access to care and medication across Europe
  - Currently chaired by professor Wiebke Arlt, President-Elect and co-chaired by PAG member organisations from the UK, Sweden and the Netherlands

International congresses: medicines shortages project

- Launched during ESE congress in Stockholm
- 3 versions:
  - Member organisations of ESE (National Societies)
  - Endo-ERN Reference centers
  - Patient organisations (not the members)

Research and Surveys

- Teaming up with clinicians, researchers and PhD candidates
- Great opportunity to deliver the unmet need of patients in the right place
- Learning opportunity to master the survey instrument
- Challenge to reach all relevant patients: reaching mostly the ‘usual suspects’



> Endocrine. 2021 Mar;71(3):561-568. doi: 10.1007/s12020-021-02618-z. Epub 2021 Feb 3.

Patients with rare endocrine conditions have corresponding views on unmet needs in clinical research

Johan P de Graaf [#, 1], Friso de Vries [#, 2], Anne Dirkson [3], Olaf Hiort [4], Alberto M Pereira [5], Márta Korbonits [6], Martine Cools [7], Research and Science Work Package of Endo-ERN

Affiliations + expand

PMID: 33534110 PMCID: PMC8016771 DOI: 10.1007/s12020-021-02618-z

Free PMC article

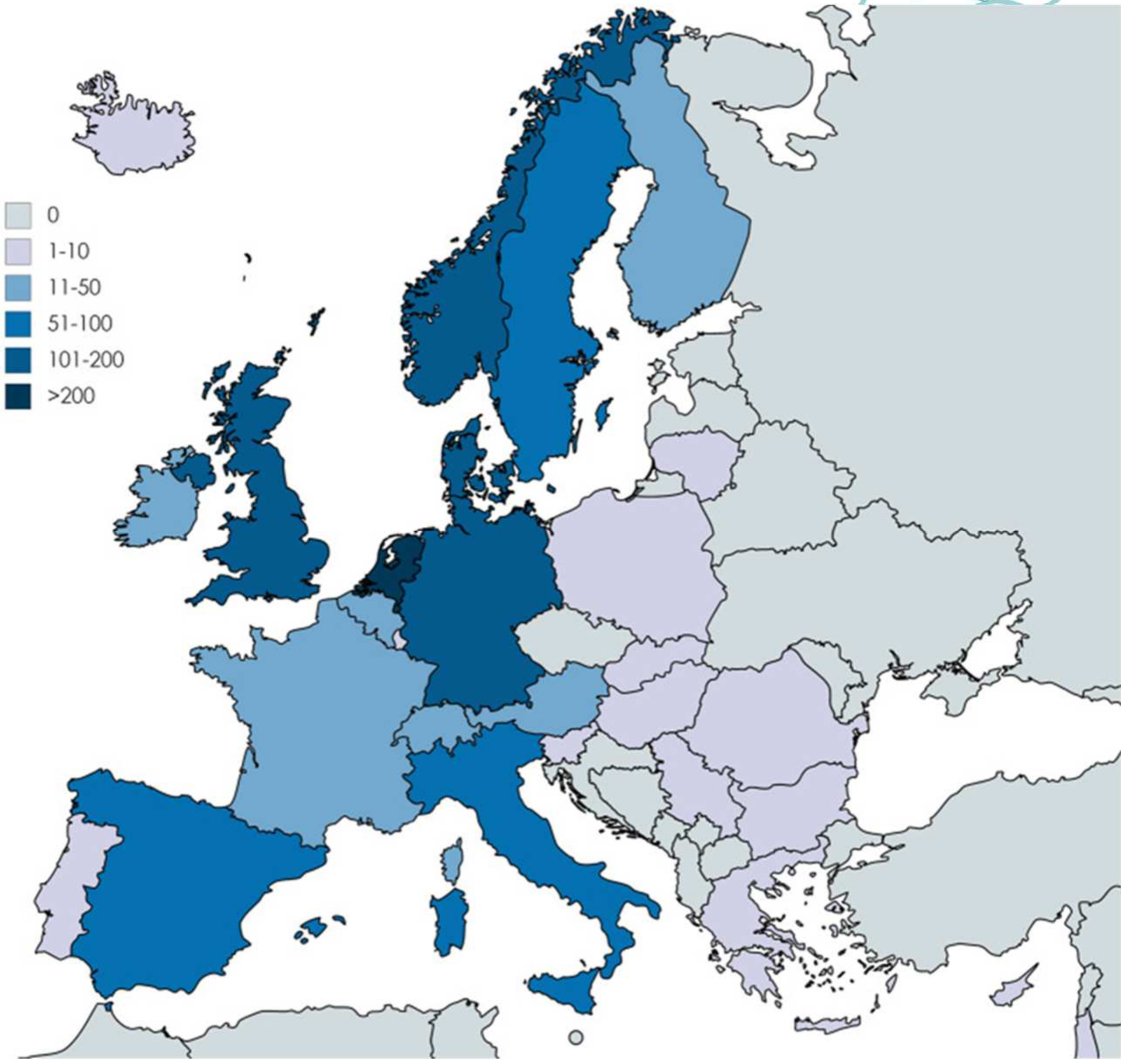
Abstract

Purpose: European Reference Network on Rare Endocrine Conditions' (Endo-ERN) mission is to reduce and ultimately abolish inequalities in care for patients with rare endocrine conditions in Europe. This study assesses which themes related to rare endocrine conditions are prioritized by patients for clinical research.

Research and surveys

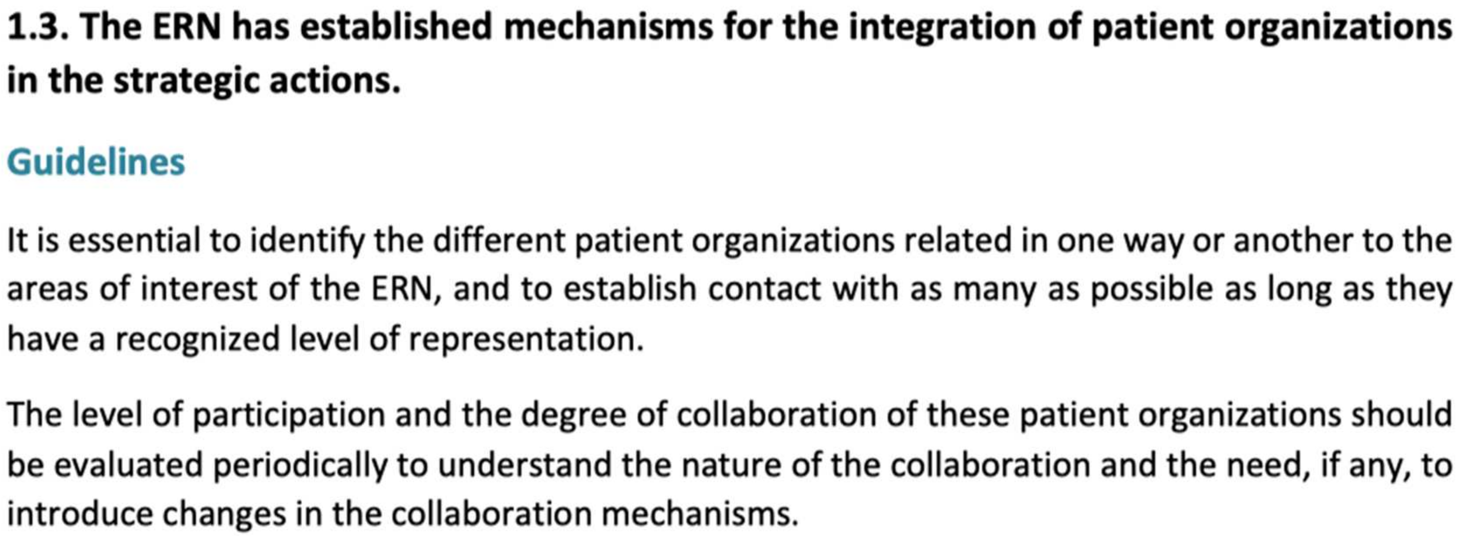
- Generating neutral questions: ‘What’s keeping you awake at night?’
- The Northwestern European bias
- How to cope with a lack of patient representation
- Just a translation isn’t enough…

- It is so important to incorporate the voice of all patients in Europe!
- New surveys coming up: help needed!!



Measuring Patient Involvement within Endo-ERN

- Part of the ERN evaluation is to monitor how patient involvement is organised:



1.3. The ERN has established mechanisms for the integration of patient organizations in the strategic actions.

Guidelines

It is essential to identify the different patient organizations related in one way or another to the areas of interest of the ERN, and to establish contact with as many as possible as long as they have a recognized level of representation.

The level of participation and the degree of collaboration of these patient organizations should be evaluated periodically to understand the nature of the collaboration and the need, if any, to introduce changes in the collaboration mechanisms.

European Commission

ERNs EVALUATION RESULTS REPORT

Independent Evaluations of European Reference Networks and of Healthcare Providers

Contract No HADEA-2022-P3-04

Report submitted by the Consortium:

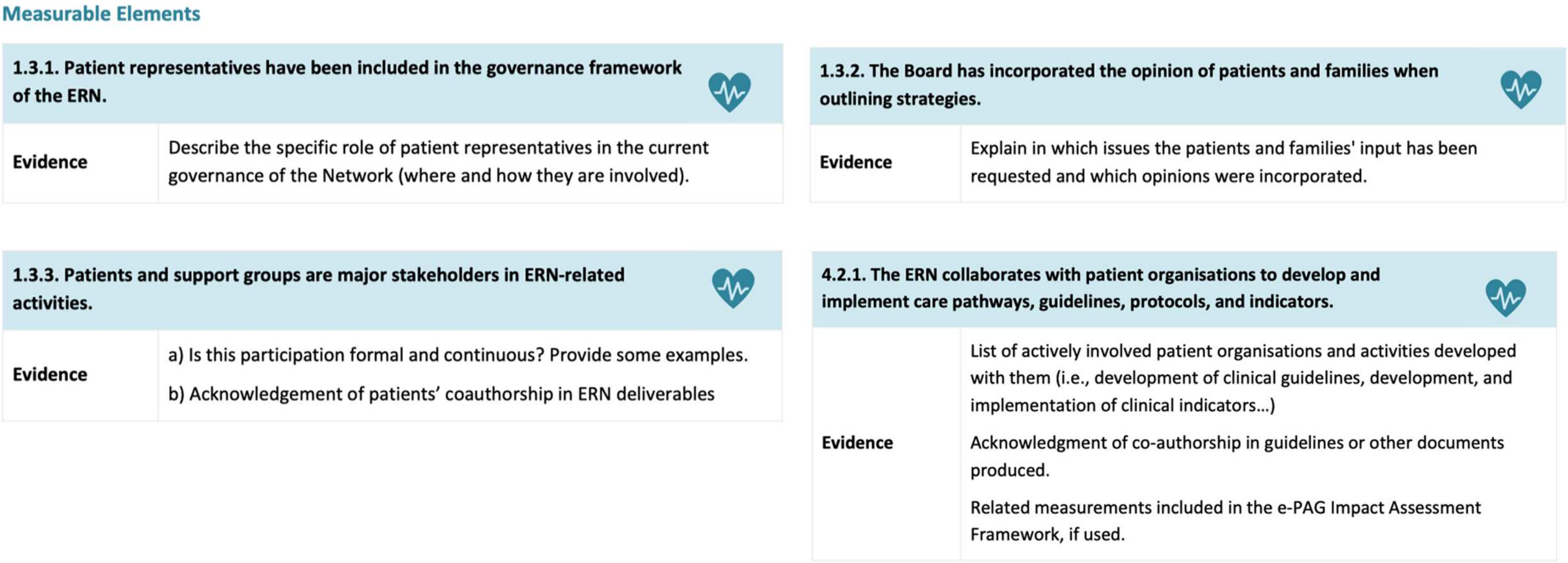
IDOM, Consulting, Engineering, Architecture, S.A.U.

and Fundación Pública Andaluza Progreso y Salud

EUHealthSupport Consortium

October 2024

Measuring Patient Involvement within Endo-ERN (II)



Measurable Elements

1.3.1. Patient representatives have been included in the governance framework of the ERN.	
Evidence	Describe the specific role of patient representatives in the current governance of the Network (where and how they are involved).

1.3.2. The Board has incorporated the opinion of patients and families when outlining strategies.	
Evidence	Explain in which issues the patients and families' input has been requested and which opinions were incorporated.

1.3.3. Patients and support groups are major stakeholders in ERN-related activities.	
Evidence	a) Is this participation formal and continuous? Provide some examples. b) Acknowledgement of patients' coauthorship in ERN deliverables

4.2.1. The ERN collaborates with patient organisations to develop and implement care pathways, guidelines, protocols, and indicators.	
Evidence	List of actively involved patient organisations and activities developed with them (i.e., development of clinical guidelines, development, and implementation of clinical indicators…) Acknowledgment of co-authorship in guidelines or other documents produced. Related measurements included in the e-PAG Impact Assessment Framework, if used.

Measuring Patient Involvement within Endo-ERN

Example of ePAG Petra Brügmann, MTG 4 Genetic Endocrine Tumour Syndromes

Date	Name of project	Contribution	Impact
February 2024	S/A Meeting	• Slides with results of EMENAs MEN survey	• Patients' views on unmet needs shared to inform discussion.
May 2024	General Assembly, Milan	• Contribution to the MTG 4 Session & Presentation	• Patients' views on guidelines and registries.
May 2024	EuRRECa	• Contribution to symposium, poster and presentation • Insisting on MEN 1 module	• Presenting patient views' view on registries as results of the MEN survey. • It is in discussion and will be delivered.
23 May 2024	Pathways	• Start of the discussion on MEN 1 Patients' Journey & Pathway.	• Insisting on the topic
01 May 2024	ESE/ESPE/EndoERN/ePAG	• Survey on drugs and medicines shortages, three different drafts for the different groups (ESC national societies, ESPE national societies, European PO with endocrine conditions.	Discussion with ESE and Johan de Graaf – compiling the drafts into an EU survey tool.

Being prepared as a patient representative: Training opportunities

- Eurordis Open Academy
  - EURORDIS Open Academy School on Medicines Research & Development
  - EURORDIS Open Academy School on Scientific Innovation & Translational Research
  - Leadership & Advocacy training
  - The EURORDIS Digital School on Social & Digital Media
- European Patient's academy on therapeutic innovation (EUPATI)
  - Toolbox; table of contents with references to relevant parts of the course material
  - Open Classroom (online course modules)
  - European version and a French version
- EUCAPA; being prepared for HTAR (Health Technology Assessment Regulation)
  - Introductory trainings on HTAR
  - Fast track HTA trainings for Joint Clinical Assessment, Joint Scientific Counsiltations)
  - Extended HTA trainings

Thank you for your attention

johan.degraaf@hypofyse.nl

11EME JOURNÉE ANNUELLE FIRENDO

MERCREDI 4 DÉCEMBRE 2024 PARIS 14e

FIRENDO

FILIÈRE MALADIES RARES ENDOCRINIENNES