European Reference Network

Endo-ERN

Setting the scene for Endo-ERN

prof. Alberto Pereira Coordinator Endo-ERN, Adult chair

Adult Endocrinologist

Amsterdam UMC (Amsterdam, Netherlands)

Alberto Pereira

prof. dr. Olaf Hiort Deputy coordinator Endo-ERN, Paediatric chair

Paediatric Endocrinologist

Universitätsklinikum Schleswig-Holstein (Lubeck, Germany)

Olaf Hiort

How did Endo-ERN start?

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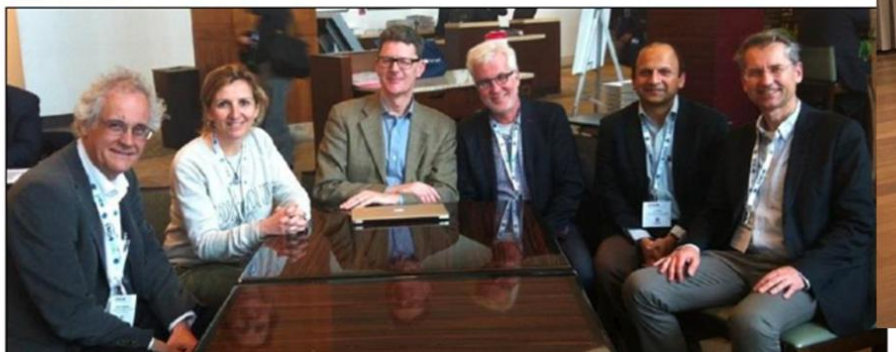
Endo-ERN

The idea of „Care across the life-span“

Leiden 2017



Boston 2016

Vilnius 2017

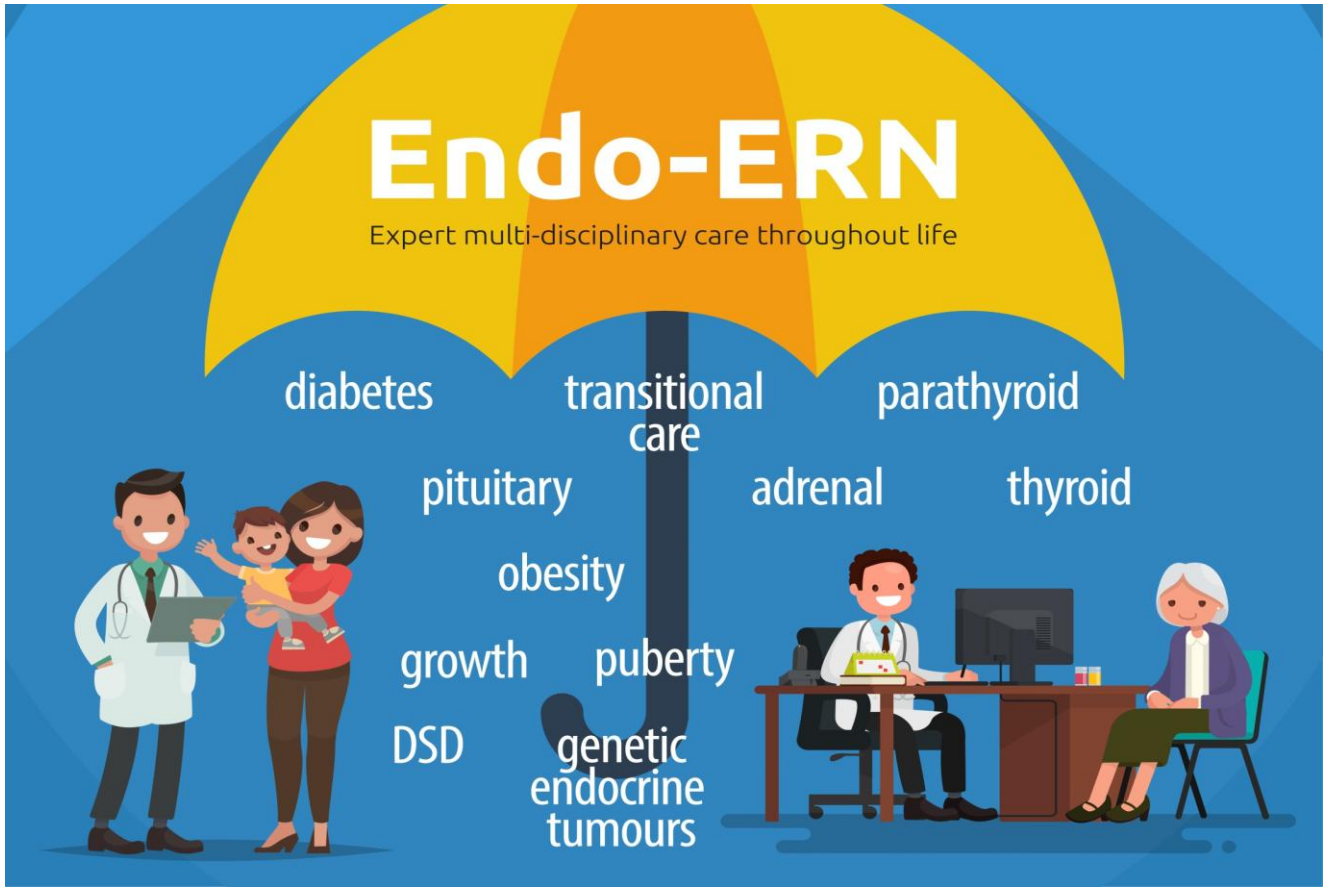
What is needed?

Elements necessary for life-long care

- **Medical perspective**
  - expertise in paediatrics
  - expertise in adult medicine
  - patient knowledge

- **Patient perspective**
  - Knowledge of the condition
  - Adapted to the life time needs
  - Access to medical expertise
  - Trust in care

- **Structural adaptation of transition**



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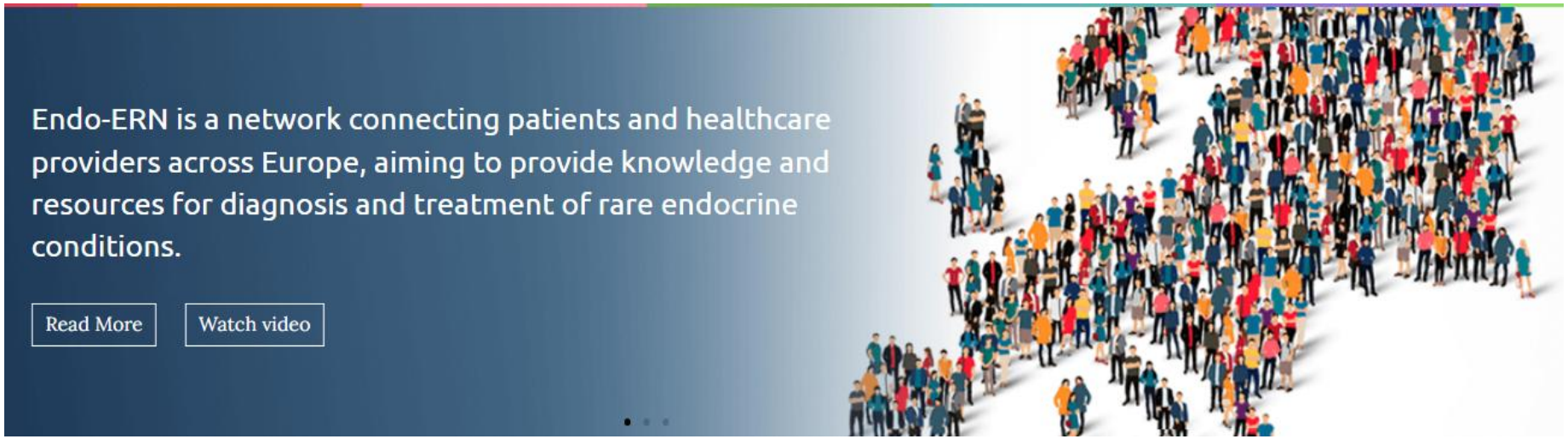
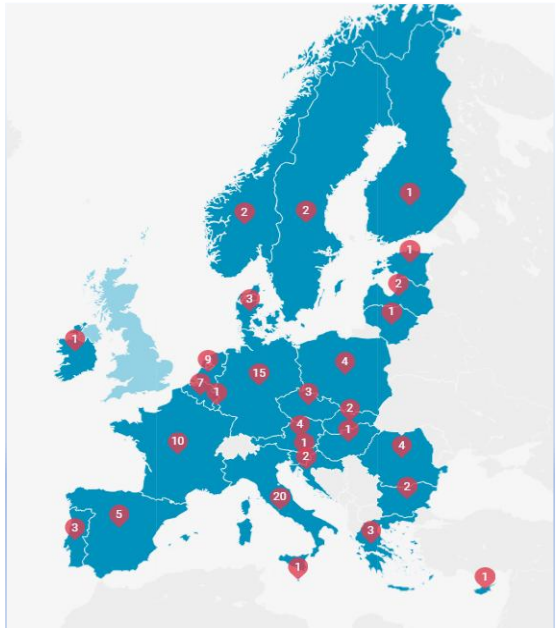
Endo-ERN

Key deliverables:

- To demonstrate added value of virtual multidisciplinary expert boards (CPMS)
- Standardization of care for rare endocrine conditions (transparency, guidelines, patient involvement)
- To demonstrate structural pathways into science



Mission: to reduce present health inequalities in Europe



Governance

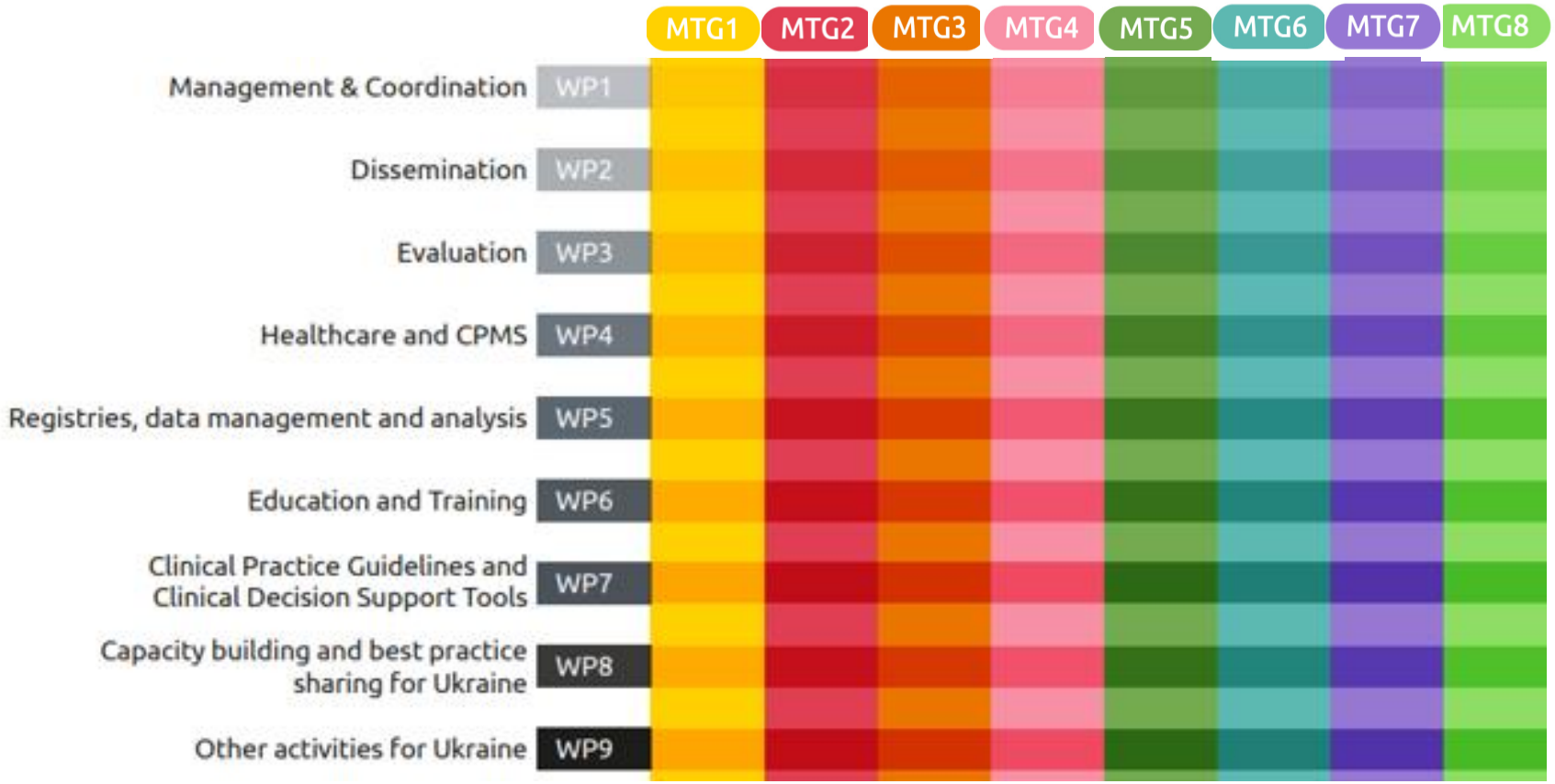

Board (Adult and Paediatric Endocrinologists)
Chair (coordinator, 1 vote)
Vice-chair (deputy coordinator, 1 vote)
Steering committee
3 chairs per Main Thematic Group and per Work Package
(Adult and Paediatric Endocrinologists and ePAG)
(1 vote per Main Thematic group and per Work Package)
Management board
(General Assembly, annual meeting)
Health Care Provider representatives
(1 per HCP, 1 vote per HCP)
National Coordinator
National representative
If issues arise linking pin
dissemination of expertise
European Commission
Advisory Board (no votes)
ESE & ESPE representatives
Representatives of other European Endocrine Societies
National representatives (National Coordinators)

Structure

Current Transversal Working Groups

Genomic Testing
Chairs: Thomas Eggermann, Dirk Prawitt

Transition in Care (Pediatric to Adult)
Chairs: Andrea Isidori , Ulla Dohnert



Endo-ERN main thematic groups

- MTG1 Adrenal
- MTG2 Disorders of Calcium & Phosphate Homeostasis
- MTG3 Genetic Disorders of Glucose & Insulin Homeostasis
- MTG4 Genetic Endocrine Tumour Syndromes
- MTG5 Growth & Genetic Obesity Syndromes
- MTG6 Hypothalamic and Pituitary Conditions
- MTG7 Sex Development & Maturation
- MTG8 Thyroid

Amsterdam Project Office

Charlotte van Beuzekom Head of Project Office

Lead: WP1, WP5, MTG3, MTG5

Other: Grant oversight and consortium management

Charlotte van Beuzekom

Jadranka Elezovic Management Assistant

Lead: WP1, MTG1, MTG6

Other: Mailbox, member contact updates, surveys

Jadranka Elezovic

Aimee Casey Communication Manager

Lead: WP2, WP6, MTG2, MTG4

Other: Social media, webinars, endorsement

Aimee Casey

Emily White

Project Manager & Operational Helpdesk Manager

Lead: WP3, WP4, WP7, MTG7, MTG8

Other: Evaluation project manager

Emily White

Grant Consortium

Work Package	Consortium member	PI name
WP1 Management & Coordination	Amsterdam UMC Universitätsklinikum Schleswig-Holstein	Alberto Pereira Olaf Hiort
WP2 Dissemination	Amsterdam UMC	Alberto Pereira
WP3 Evaluation	Amsterdam UMC	Alberto Pereira
WP4 Healthcare and CPMS	Amsterdam UMC Universitätsklinikum Schleswig-Holstein	Alberto Pereira Olaf Hiort
WP5 Registries, data management & analysis	Leiden University Medical Center	Natasha Appelman-Dijkstra
WP6 Education and Training	UMHAT “Sveta Marina"	Violeta Iotova
WP7 CPGs & CDSTs	Karolinska Institutet Leiden University Medical Center	Anna Nordenström Olaf Dekkers
WP8 Capacity building and best practice sharing for Ukraine	Amsterdam UMC	Alberto Pereira
WP9 Other activities for Ukraine	Amsterdam UMC	Alberto Pereira

What have we accomplished so far?

- Unique governance structure, with a network that is able to quickly respond when needed!
- Benchmark new cases where they are seen
- Success stories that exemplify the need for a dual chair, holistic leadership, broadness of European inclusive leadership across all member states.
- All evidenced by completed project deliverables and milestones
- Only a few examples……

Empowerment: the patient's voice

European Patient Advocacy Group

Endocrine (2021) 71:706–707
https://doi.org/10.1007/s12020-021-02653-w

LETTER TO THE EDITOR

Patient's view on better care

Jette Kristensen[1] · Manuela Brösamle[2] · Bas van den Berg[3]

Received: 25 January 2021 / Accepted: 29 January 2021 / Published online: 24 February 2021

or muscle and joint pain, but also to psychosocial problems [1] especially in patients with comorbidity. Psychological wellbeing has increasingly been the subject of research during the last decade.

The key factors for improving quality of life are early diagnosis, improved dosage of medication (3–4 times daily) and increased self-management and knowledge about the disorder for both patients and their immediate circle of carers. New medications like slow-release and modified release hydrocortisone preparations and hydrocortisone tablets with lower dosages improve and better reflect the

Diagnose → **Treat** → **Educate** → **Equip** → **Empower**

Autonomy of the patient

Quality of Life

IN MEMORIAM

Jette Kristensen

WARMTH
DEDICATION
PATIENT VOICE
FAMILY
ENTHOUSIASTIC

ADDISON FORENINGEN I DANMARK

Juliane Leger

EXPERTISE
CHILDREN
CRESCENDO
MENTOR
KINDNESS

Outcome of COVID-19 infections in patients with adrenal insufficiency and excess

Hanna F Nowotny[1], Jillian Bryce[2], Salma R Ali[2], Roberta Giordano[3,4], Federico Baronio[5], Irina Chifu[6], Lea Tschaidse[1], Martine Cools[7], Erica LT van den Akker[8], Henrik Falhammar[9,10], Natasha M Appelman-Dijkstra[11], Luca Persani[12,13], Guglielmo Beccuti[3], Ian L Ross[14], Simona Grozinsky-Glasberg[15], Alberto M Pereira[11], Eystein S Husebye[16,17,18], Stefanie Hahner[6], S Faisal Ahmed[2,11,19] and Nicole Reisch[1]

Rare Disease COVID-19 Task-force

Share This

ESE and ENDO-ERN have launched an initiative to collect data on patients with rare endocrine conditions and COVID-19, contribute your data by signing up here

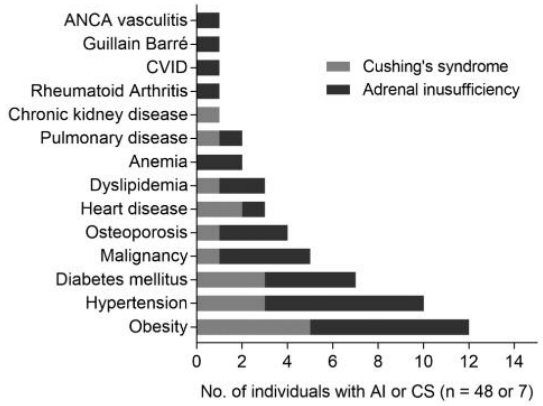
9 % hospitalized for adrenal crisis and 6% for severe infection

(1 patient in ICU)

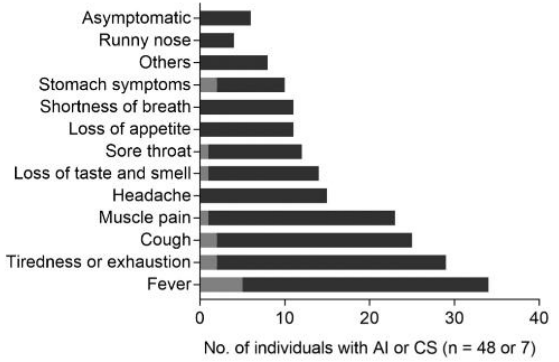
C/: good clinical outcome (provided adequate sick day rules)

Nowotny *et al*. Endocrine Connect 2023

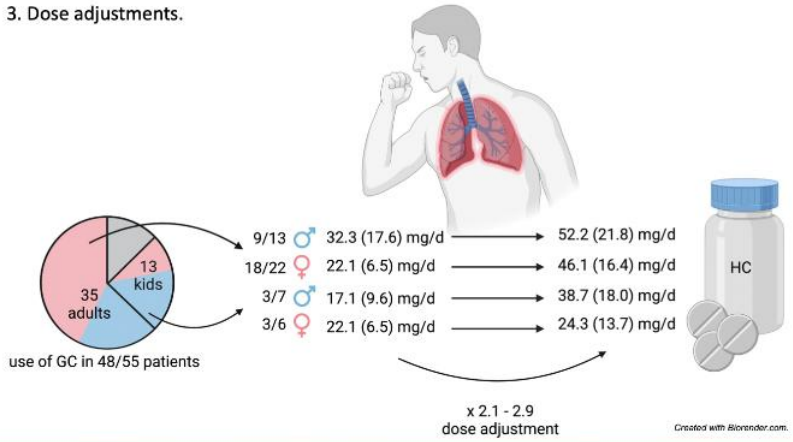
1. Comorbidities of patients with adrenal gland disorders.



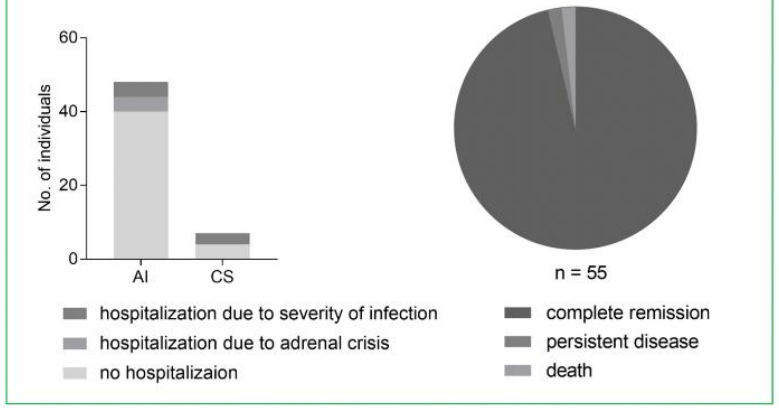
2. Symptoms of patients with COVID-19 and adrenal gland disorder.



3. Dose adjustments.



4. Hospitalization of patients with adrenal disorders and outcome of COVID-19 infection.



ERN Success stories

Endo-ERN
European Reference Netwo
on Rare Endocrine Conditio

ENDO ERN

European Reference Network on rare endocrine conditions
https://endo-ern.eu/

Members

105 healthcare providers located in 27 countries in the EU and Norway, distributed as follows:

- 91 Full Members in 21 Member States
- 11 Associated National Centres in 5 Member States and 2 in Norway
- 1 National Coordination Hub in a Member State

20 patient representatives from 10 countries are also collaborating.

Disease areas

- Adrenal
- Disorders of Calcium & Phosphate Homeostasis
- Genetic Disorders of Glucose & Insulin Homeostasis
- Genetic Endocrine Tumour Syndromes
- Growth & Genetic Obesity Syndromes
- Hypothalamic and Pituitary Conditions
- Sex Development & Maturation
- Thyroid

Guidelines, care pathways, and patient journeys

- 4 guidelines written by Endo-ERN
- About 60 guidelines co-authored and/or endorsed by Endo-ERN
- 1 care pathway
- 3 patient journeys

Training and education

- 70+ Endo-ERN webinars organised since 2019
- Joint webinar programme with European Endocrine societies (adult and paediatric)
- Endo-ERN symposium at European Endocrine Societies Annual Conferences
- 17 clinical exchanges (20 health care providers, 12 countries) since 2021
- Endorsement / accreditation of educational activities

Clinical Patient Management System (CPMS)

- 250 CPMS case discussions since 2017

Research and patients' registries

- About 200 relevant research projects or clinical trials involving at least two healthcare providers from two different Member States
- 3 850 patients recorded in the core registry (https://eurreb.eu), a shared platform with ERN BOND

What have we accomplished so far?

The EU perspective:

- Where is the economic evaluation (gain for society etc.)?
- Pathway analysis, quality assessment of the center (incl profitable from the patient perspective)
- Where is the inclusiveness of all patients with rare conditions?
- How are the network structures?
- How is caring for a lifelong care dealt with, including transition?

Where are we heading to?

Future focus

Further development of Endo-ERN, based on:

1. Integration of Endo-ERN into the national health care systems of Member States (JARDIN)

2. Integration of Endo-ERN with the European Health Data Space (EHDS) (EuRREB)

3. Integration of Endo-ERN research activities with current and future European RD research initiatives / structures (ERICA, ERDERA etc)

Positions and roles

European Society of Endocrinology

ESE RD Committee

(co)Chairs	Term
Elena Valassi (ESE)	2024 – 2028
Alberto Pereira (Endo-ERN)	2019 – 2027
Endo-ERN representatives	
Gudmundur Johannsson	2020 – 2026
Marek Niedziela (ESPE)	2024 – 2028
Luca Persani	2024 – 2028
Dimitra Vassiliadi	2024 – 2028

Successor Olaf Hiort

European Journal of Endocrinology, 2025, **193**, ii128–ii131
https://doi.org/10.1093/ejendo/lvaf072

Review Article

EndoCompass project: rare diseases in endocrinology

Alberto M. Pereira[1,2,*] **and Olaf Hiort**[2,3]

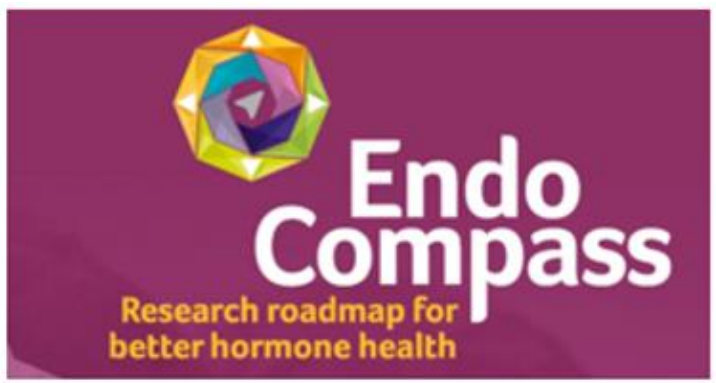
[1]Department of Endocrinology and Metabolism, Amsterdam University Medical Center, Meibergdreef 9, 1105AZ Amsterdam, the Netherlands
[2]European Reference Network on Rare Endocrine Conditions (Endo-ERN)
[3]Sektion für Pädiatrische Endokrinologie und Diabetologie, Klinik für Kinder- und Jugendmedizin, Universität zu Lübeck, Ratzeburger Allee 160, Lübeck 23562, Germany

*Corresponding author: Department of Endocrinology and Metabolism, Amsterdam University Medical Center, Meibergdreef 9, 1105AZ Amsterdam, the Netherlands. Email: a.m.pereiraarias@amsterdamumc.nl

Focus areas for research into rare conditions

The following are examples of specific topics that should be considered when deciding on a research grant. They have been incorporated into the Endo-ERN Quality of Care Improvement and Research Counsel:

1. Diagnostic research readiness:
   - Assessment of data readiness, standardization, and harmonization
   - Genome re-analysis pipeline application, variant interpretation, and translation to endocrinology departments or clinics
   - Genomic innovation to shorten time to diagnosis
2. Clinical trial readiness:
   - Multinational patient inclusion and collaboration
   - Disease progression modelling
3. Clinical outcome assessment:
   - Estimating the socio-economic impact of rare endocrine diseases
   - Digital tool and pathway development
   - Develop/implement disease-specific clinical outcome assessment tools
4. Innovative therapies:
   - Advanced therapeutic medicinal products need assessment/mapping in Endo-ERN
   - Analysis of specific therapy and treatment availability across EU member states
5. Unique clinical resource development/pilot:
   - Disease-specific patient journey development
   - Rare endocrine disease patient pathway development/application
   - Mapping of European patient referral pathways/channels in Endo-ERN



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The need for a Transversal Research WG

Implement ***Focus areas for research into rare conditions***

- Map research landscape within Endo-ERN and the wider ERN ecosystem
- Raise awareness of persistent underfunding of endocrine research despite its major impact on the EU population
- Highlight patient impact and unmet needs in rare endocrine conditions

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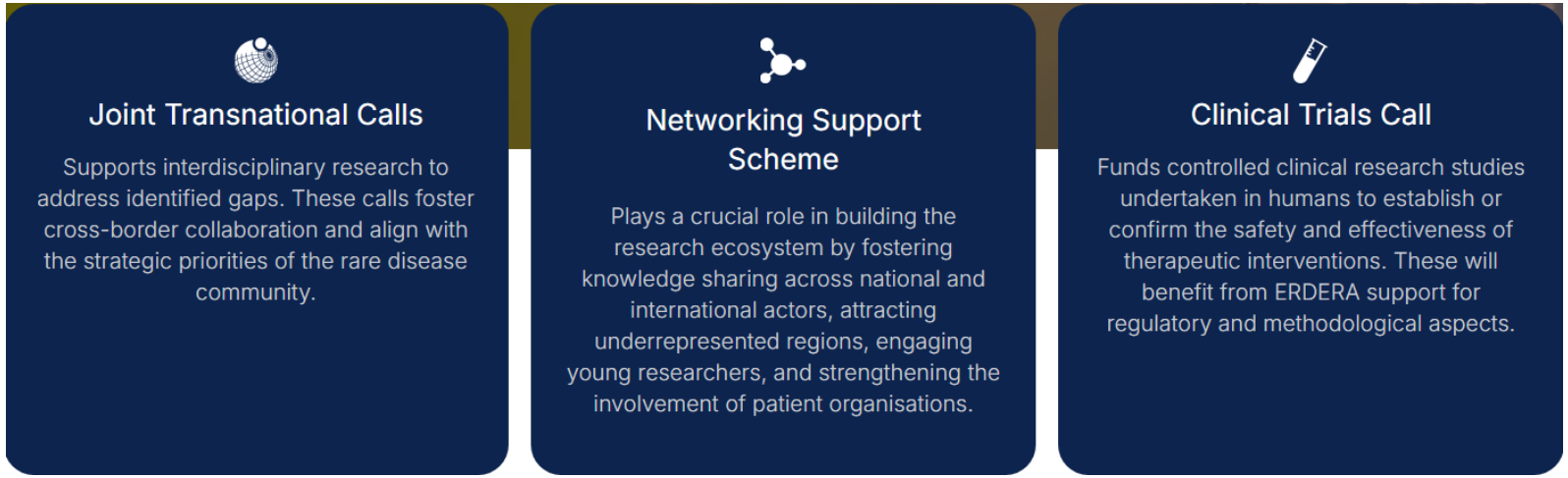
ERDERA

www.erdera.org/funding



Endo-ERN

- 5 pre-proposals
- full proposals upon invitation
- deadline 8 July

- 2 submissions
- 1 selected for funding (TALENT)

The first ERDERA Clinical Trials Call will be soon available.

Funding grants

Endo-ERN coordination grant (EU4Health)

- 1 Oct 2023 – 1 Oct 2027
- Interim Report & Project Review Meeting
- Future funding

Funded by the European Union

FIRENDO x Endo-ERN

- Grant for 2026-2027
- Collaborative research project
- FIRENDO member + Endo-ERN HCP (outside France)
- Max Euro 60.000
- Deadline May 3rd, 2026

www.firendo.fr/en/call-for-research-projects-europe-2025

Journals – special issues & collaboration

Endocrine

- Special edition 2021

Endocrine Connections

- Special issues 2022-2026
- Special issue 2027/2028?
- Joined webinars
- Endorsement & collaboration proposal

Endocrine Oncology

- Special issue 2026/2027
- Topic suggestions
- Next steps

European Reference Network

Endo-ERN

How do YOU see our ERN?

Do you like the dual structure?

What is profitable to the individual member?

Should we distribute a short questionnaire?


Current Endo-ERN grant
Athens
April 2026
GA April/May 2027
Preparation for
new grant preferably
with new pediatric chair
Spring 2027
End of
Current grant
1 Oct 2027
New Grant
DUAL chair