ERN alignment with ERDERA & RealiseD and JARDIN

Endo-ERN General Assembly 21-4-2026

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Opportunities for funding & Support

- **ERICA**: European Rare Disease Research Coordination and Support action CSA (for ERNs) (funding ended sept 2025);
- **ERDERA:** European Rare Disease Alliance (pan-European RD Science Partnership)
  - WP25 Task 25.4 Alignement with Research Strategies of the ERNs
- **RealiseD:** Advancing Clinical Trials for (Ultra) Rare Diseases

Realise D

compRehensive mEthodological and operational Approach to cLinical trIalS in ultra-rarE Diseases

JARDIN

European Reference Network

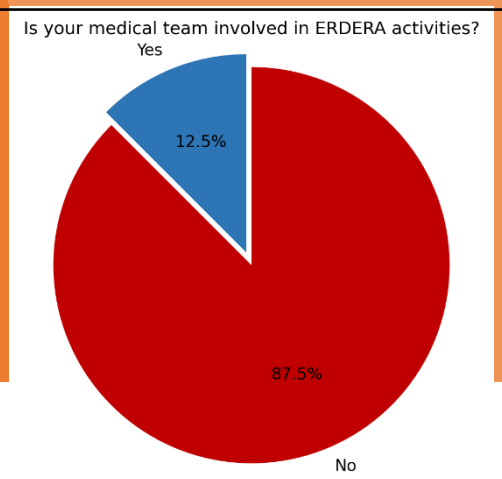
Joint Action on integration of ERNs into national healthcare systems -JARDIN

European Commission

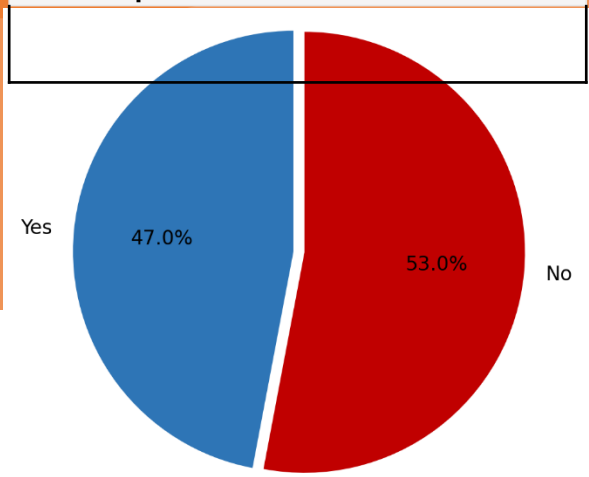
ERDERA WP25.4 Survey on ERN Participation & Collaborative research projects

- 🎯 **Purpose: Gather information on clinical research across ERNs and HCP involvement in ERDERA**
- 👥 **Target Audience: ERN Coordinators and HCP Members**
- 📊 **Results : 168 responses from all**

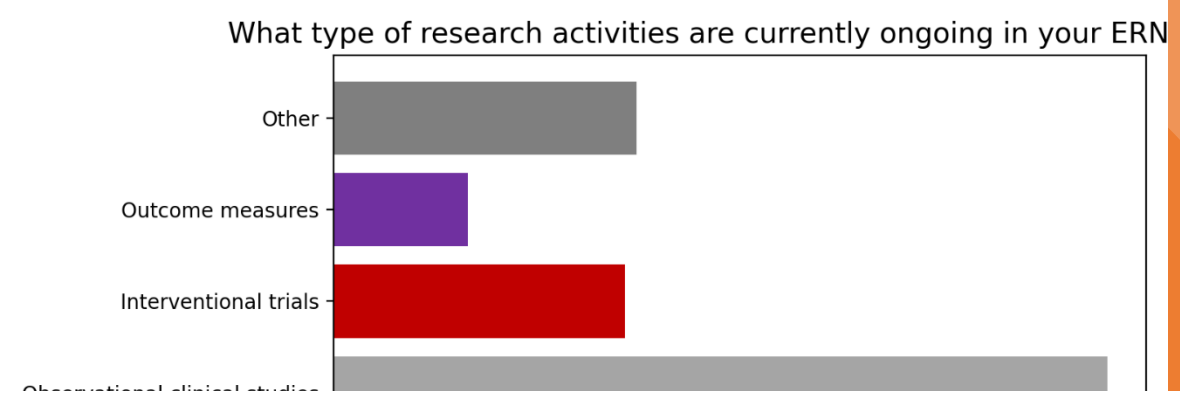
Endo-ERN has transversal research WGs in place or under development, but visibility and operational reach vary considerably across centres.



Does your ERN/HCP have a transversal Working Group for research?



Survey Results:



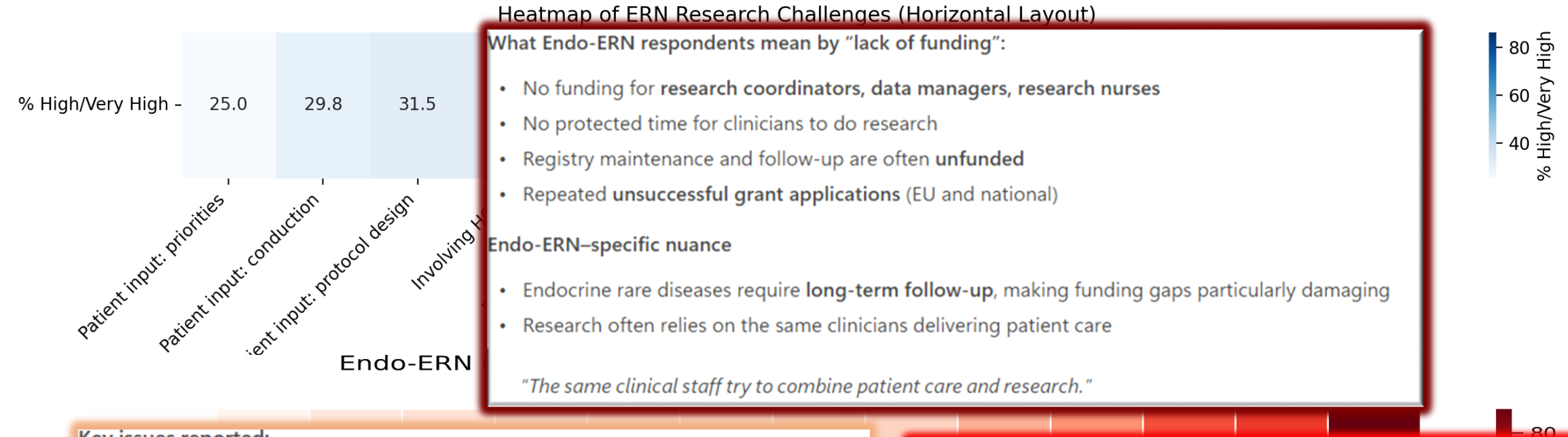
What do you think are the most eminent topics for ERN research in the coming 5 years (rank at least top 3):

Positie	Opties	Endo-ERN priority distribution
1	Patient-centred outcome measures	
2	Pragmatic (registry-based) clinical trials	
3	Omics / biomarker expertise sharing	
4	Harmonised data capture & FAIRification	
5	Genomic diagnostic expertise sharing	
6	Natural history studies	
7	Investigator-initiated trial execution	
8	Advanced experimental therapies	
9	Creation of biorepositories	
10	Legal issues (GDPR, Informed Consent)	

Other (registries & platforms) Strong transversal backbone across activities

Positie	Opties	Eerste keuze … Laatste keuze
1	Patient-centred outcome measures	
2	Pragmatic (registry based) clinical trials	
3	Omics / biomarker research expertise sharing	
4	Harmonized data capture (incl. collection/storage and data FAIRification)	
5	Genomic diagnostic expertise sharing	
6	Legal issues (data protection, Informed Consent)	
7	Natural History studies	
8	Investigator initiated trial planning & execution	
9	Advanced experimental therapies	
10	Creation of biorepositories	

Survey Results: Challenges



What Endo-ERN respondents mean by "lack of funding":

- No funding for **research coordinators, data managers, research nurses**
- No protected time for clinicians to do research
- Registry maintenance and follow-up are often **unfunded**
- Repeated **unsuccessful grant applications** (EU and national)

Endo-ERN–specific nuance

- Endocrine rare diseases require **long-term follow-up**, making funding gaps particularly damaging
- Research often relies on the same clinicians delivering patient care

"The same clinical staff try to combine patient care and research."

Key issues reported:

- Non-interoperable EHR systems across countries
- Manual data entry and consent management
- Long-term sustainability of registries
- Data quality vs workload trade-offs

"Registries exist but are hard to sustain."
"Data collection is time-consuming and unfunded."

Endo-ERN–specific nuance

- Endocrine diseases often evolve slowly → need **longitudinal high-quality data**
- Transition of care (paediatric → adult) complicates data continuity

Endo-ERN respondents repeatedly describe **regulatory burden as a bottleneck**, especially for and investigator-initiated studies.

Concrete issues mentioned:

- Long timelines for ethics approval
- National differences in regulatory requirements
- CTIS submissions without institutional guidance
- GDPR, consent wording, and translation burdens

"Very difficult to accomplish approval of a multicentre study."
"No guidance from the centre when submitting to CTIS."

Endo-ERN–specific nuance

- Many Endo-ERN studies are **international by necessity**, amplifying regulatory complexity
- Smaller patient numbers mean approval delays are disproportionately damaging

Clinical trials: challenges and solutions

Clinical trials need centralised European support, not just local effort.

❗ Key challenges (Endo-ERN responses)

- Recruitment of rare endocrine patients
- Lack of time and protected research capacity for clinicians
- Insufficient personnel (research coordinators, study nurses)
- Complex and lengthy regulatory and ethics procedures
- Fragmented administrative support across centres
- Funding constraints and failed grant applications

"Very difficult to accomplish multicentre trials."

"Lack of time of clinicians to include patients."

✅ Proposed solutions (from Endo-ERN respondents)

- Establish a **European structure** to support:
  - trial development
  - feasibility assessment
  - regulatory submissions (incl. CTIS)
- Provide **dedicated research coordinators / administrative staff** per centre
- Allocate **protected research time** for clinicians
- Harmonise and simplify administrative and ethics procedures
- Increase **targeted funding** for investigator-initiated trials
- Strengthen **cross-country recruitment** through coordinated Endo-ERN efforts

Natural history studies: challenges and solutions

Natural history studies require sustained, structured infrastructure rather than project-based solutions.

❗ Key challenges

- Long follow-up periods for prospective studies
- Retrospective datasets often incomplete or heterogeneous
- Small patient populations per country
- Limited time during clinical visits to collect high-quality data
- Lack of staff dedicated to longitudinal data collection

"Short visits do not allow collection of all relevant data."

✅ Proposed solutions

- Develop **high-quality, standardised databases** for natural history
- Facilitate **multicentre and cross-country collaboration**
- Provide **dedicated personnel** for data collection and follow-up
- Allocate funding for **long-term data collection**
- Improve **family and patient involvement** in long-term studies
- Leverage **large medical datasets** where possible for longitudinal input

Registries: challenges and solutions

❗ Key challenges

- Maintenance costs of registries
- Regulatory and GDPR complexity
- Data-sharing barriers between countries
- Manual and paper-based consent procedures
- Lack of interoperability between national EHR systems
- Insufficient staff to manage registries

"Registries exist, but are hard to sustain."

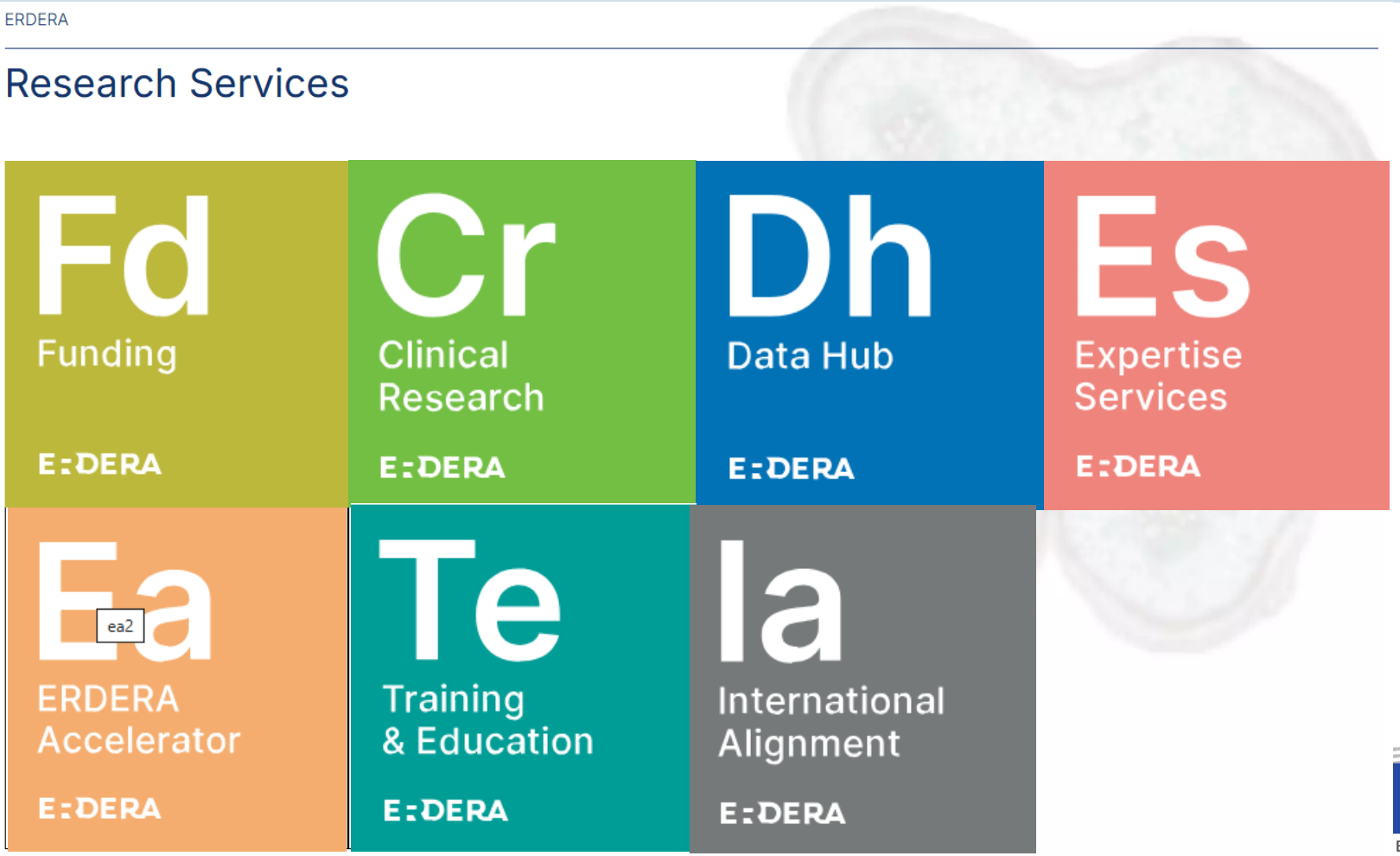
Registries are the backbone of Endo-ERN research and require long-term European commitment.

✅ Proposed solutions

- Develop **European shared, high-quality web-based registry platforms**
- Promote **shared registries** across ERNs and countries
- Implement **standardised European data-sharing rules**
- Introduce **online validated consent procedures**
- Secure **structural funding** for registry maintenance
- Provide **helpdesk support** for clinicians and centres
- Enable **central endorsement by health authorities**, where possible

ERDERA- how to navigate

[Homepage - ERDERA]



European Reference Network

European Commission

Joint Transnational Calls

Supports interdisciplinary research to address identified gaps. These calls foster cross-border collaboration and align with the strategic priorities of the rare disease community.

Networking Support Scheme

Plays a crucial role in building the research ecosystem by fostering knowledge sharing across national and international actors, attracting underrepresented regions, engaging young researchers, and strengthening the involvement of patient organisations.

Clinical Trials Call

Funds controlled clinical research studies undertaken in humans to establish or confirm the safety and effectiveness of therapeutic interventions. These will benefit from ERDERA support for regulatory and methodological aspects.

Fd
Funding

E:DERA

ERDERA will launch **seven Joint Transnational Calls** co-funded by the European Commission

CLOSED CALL
Joint Transnational Call 2026

CLOSED CALL
Joint Transnational Call 2025

Active & Upcoming Calls

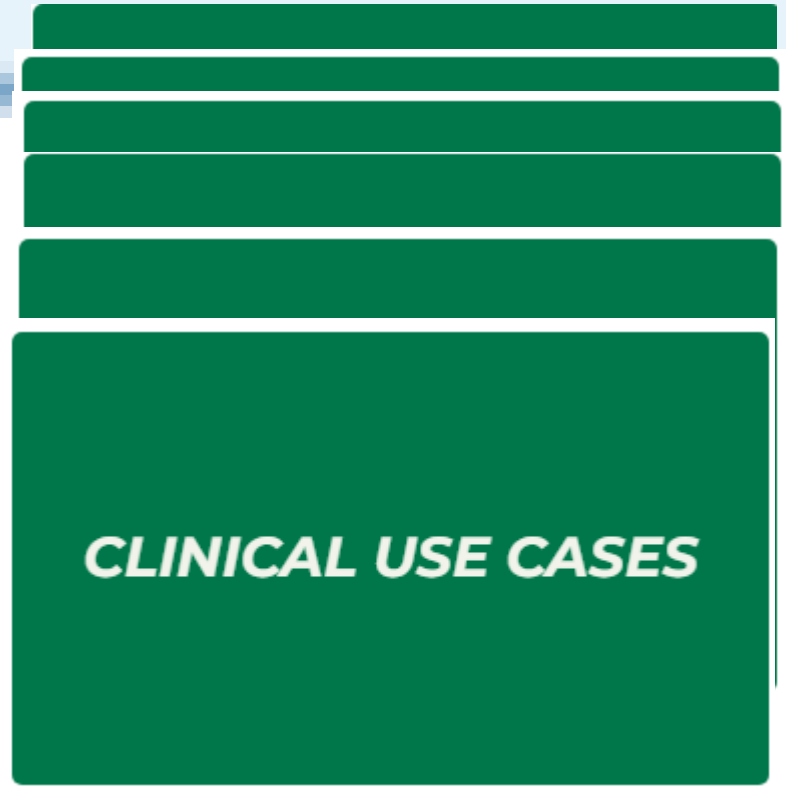
OPEN CALL
Networking Support Scheme
The current submission round ended on 7 April/ Next submission deadline 7 October 2026

OPEN CALL
Clinical Trials Call
Upcoming end 2026

European Reference Network

European Commission

RealiseD https://realised-ihi.eu/



Innovative clinical trial designs

Innovative analysis and data use strategies

Regulatory and HTA dialogue

Multistakeholder co-creation Implementation and deployment

Improve clinical trial execution

Clinical Use Cases

Learn from real clinical cases what counts and can help in other (ultra) rare diseases

CHALLENGE

Develop innovative patient aligned outcome measures, advanced statistical models and innovative trial designs require concrete data and testing.

SOLUTION

Select **4 cases** studies from different clinical areas- rare blood, bone, epilepsies and eye diseases, to:

- **Identify** clinical trial design and data challenges
- **Establish** informative and patient-centered endpoints*
- **Test** innovative clinical trial methodologies that can be used across rare diseases

* Endpoints: Clinical trial endpoints are outcome measures used to determine if the trial was successful or not

This project is supported by the Innovative Health Initiative Joint Undertaking (IHI JU) under grant agreement No 101165912. The JU receives support from the European Union's Horizon Europe research and innovation programme and COCIR, EFPIA, Europa Bío, MedTech Europe, and Vaccines Europe.

RealiseD https://realised-ihi.eu/

IMPROVE CLINICAL TRIAL DESIGN and EXECUTION	STATISTICAL INNOVATION	REGULATORY and HTA DIALOGUE
MULTI-STAKEHOLDER COLLABORATION	PATIENT- CENTRICITY	CLINICAL USE CASES

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Webinars - Realise D

RealiseD: Realising Clinical trials in Ultra-Rare diseases

Evidence Assessment Framework. The need for a mindset shift among developers, regulators and HTAs

Single arm, RCT or something in between – how to enrich clinical trial design and analysis in rare diseases

Enhancing Patient-Centricity in Rare Disease Clinical Trials

European Reference Network

Joint Action on integration of ERNs into national healthcare systems - JARDIN

JARDIN will develop strategies for systematic dissemination of information on the ERNs, with a specific emphasis on people living with rare diseases as well as the healthcare professionals community.

Co-funded by the European Union

Input via Surveys:

Topic 1.: Working conditions of the ERN centre

Topic 2.: Staff education and research activities

Topic 3.: ERN centre recognition

Topic 4.: Funding model

Topic 5.: Quality of care

Topic 6.: Centre development strategies

Topic 7.: Structure and Findability of Rare Disease Expert Centers

Topic 8.: National RD Data Management Policies

Jardin examples of results

As the reuslts are not yet officcially published, we cannot share them.

Jardin
What next?

- Recommendations formulated
- Presented to
  - National representatives
  - Hospital managers representatives
  - ERN Representatives
  - Patient representatives
  - Board of Member States

Publications and reports to draw attention and to support further ERNs on National and Hospital level.