European
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14th Meeting of the ERN Coordinators Group

30.04.2021 10:00 – 17:00 CET

European Commission
Directorate-General for Health and Food Safety
Unit B3 - Digital Health, European Reference Networks

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Housekeeping rules

1. Please identify yourself using first and last name and affiliation
2. Switch off camera and mute microphone when you are not speaking
3. Use the chat box for questions, remarks or requesting the floor

The chat transcript will be copied to the minutes.
The session is being recorded to help writing the minutes - let us know if you have a problem with recordings.

Thank you for your cooperation!

Agenda

9:45 – 10:00 Dial-in		
1.	**10:00 – 10:05**	**Welcome, approval of the agenda and minutes of past meeting**
2.	**10:05 – 10:20**	**Update from the internal ERN-CG meeting**
3.	**10:20 – 10:40**	**Update from the ERN Board of Member States**
4.	**10:40 – 10:50**	**Enlargement of the Networks – on-going call for new ERN members**
5.	**10:50 – 11:05**	**Potential new ERNs**
6.	**11:05 – 11:20**	**Future direct ERN grants**
7.	**11:20 – 11:40**	**Evaluation of Cross-border healthcare directive**
11:40 – 11:55 Coffee break		
8.	**11:55-12:15**	**Managing disease areas within existing Networks**
9.	**12:15-12:35**	**Review of legislation on orphan medicinal products**
10.	**12:35-12:40**	**Update on AMEQUIS project**
11.	**12:40-12:50**	**Potential involvement of ERN coordinators in defining future projects**
12.	**12:50-13:00**	**ERN Working Groups and their reporting – general issues**

13:00 – 14:00 Lunch Break		
13.	14:00 – 14:15	Report from Working group on ERN integration
14.	14:15 – 14:35	Report from Working group on monitoring
15.	14:35 – 15:00	Report from Working group on knowledge generation
16.	15:00 – 15:10	Report from Working group on legal and ethical issues
15:10 – 15:30 Coffee break		
17.	15:30 – 15:50	Report from ERNs IT Advisory Group (ITAG)
18.	15:50 – 16:00	CPMS activity report
19.	16:00 – 16:20	Report from Task Force on ERN registries
20.	16:20 – 16:40	European Health Data Space and ERN registries
21.	16:40 – 16:50	Any Other Business
17:00 End of meeting		

Update from the internal ERN-CG meeting

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Update from the Internal ERN-CG meeting

Prof. Nicoline Hoogerbrugge, MD, PhD
ERN GENTURIS
On behalf of all ERN coordinators

30 April 2021

Radboudumc

Changes in the chair-team

- Current chair team

Chair
Nicoline Hoogerbrugge

Vice chair
Helene Dollfus

Past Chair
Irene Mathijssen

Changes in the chair-team

- Current chair team changes per 16 June 2021

Chair
Helene Dollfus

Vice chair
Elected on 29 April

Past Chair
Nicoline Hoogerbrugge

Enlargement of the ERN Networks

Thank you very much for your cooperation and agreeing of the BoMS to almost all decisions from the ERN Boards on potential full members.

Financial support

ERNs are growing from starters into adulthood:

- In size, due to Affiliated Partners and new Full Members
- By intensifying key actions: CPMS, cross-border health care
- By new management tasks: monitoring, assessment procedures
- By expansion of activities: registries, research, teaching activities

The current financial support is no longer sufficient.

In Nov 2020 we presented our top 5 financial priority list.

This list is unchanged and still appropriate

Financial support

Questions from the ERN coordinators:

- What is the state of play in making the financial situation more sufficient and increase the budget?
- How about increasing the financing rate from 60% to 100%?

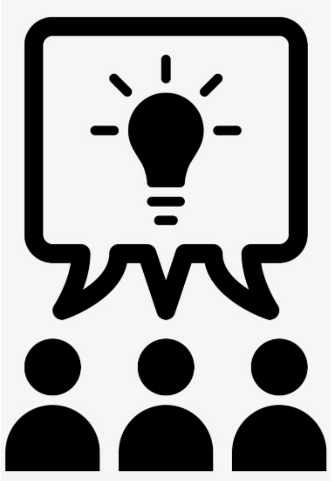
Essential to have clarity before Dec 2021 (contracts current project managers).

N. Hoogerbrugge on behalf of all ERN coordinators

Working Groups

- Co-chaired by an ERN coordinator
- WG include a few representatives from a few ERNs
- WG includes a few representatives of the BoMS
- WG Representatives of the ERNs report in our biannual meetings.

Question: Are we on the same page?



ERN Registries

For all ERN registries there is a need of uploading data form local medical files.

Challenges at local and at National level:

- Technical
- Legal
- Ethical

Question: How can these problems be addressed at a European and at a national level

Is there a role for the BoMS?

N. Hoogerbrugge on behalf of all ERN coordinators

Monitoring

- The challenge is to show what the ERNs are achieving in a lean and mean way.

Question:
How can we keep the monitoring and assessment procedures Lean and Mean?

Future Tenders to support the ERN

- ERN coordinators are willing to support the commission in making the most needed and most effective tenders.

E.g. the 3 previous tenders:

- 1) Defining the catalogue of services in an ERN (172k) (*outcome unclear*)
- 2)Taxonomy (85k) (*outcome not user-friendly*)
- 3)support in writing Guidelines (3.756k) (*focus rearrangement to unmet needs*)

Question: How can the ERN be involved in the development of a tender?

N. Hoogerbrugge on behalf of all ERN coordinators

CPMS

What is the current position of OPEN app?

e.g. we need to consult Open App on basic questions but they do not respond on

- For uploading of data in CPMS from local EPDs
- Requirements for generic connection between local EPD system and CPMS

Process for new affiliated partners in not yet covered member states

- After the procedure for new members is finished in July 2021, only a few EU member states will not yet be covered.

Question:
What is the timeline for a **new procedure for affiliated partners** in not yet covered member states?

… ERNs are growing from starters into adulthood …

Thank you fo making this possible!

Update from the ERN Board of Member States

Information point

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European Reference Networks Coordinators Group (ERN – CG) 14th Meeting

Update from the ERN Board of Member States

Till Voigtländer

30. April 2021

Contents

- Novelties
  (conceptual stage)
- Requests targeted to the Board
- Further results of the BoMS meeting

Novelties 1

- Establishment of internal BoMS meetings (following the example of the ERN-CG)
  - Room for general reflection
  - Room for reflection on actual „ad-hoc“ topics

Novelties 2

- Establishment of internal BoMS meetings (following the example of the ERN-CG)
  - Room for general reflection
  - Room for reflection on actual „ad-hoc“ topics
- Establishment of a regular exchange between the Board and the CG co-chair level ahead of official Board- and Coordinators Group meetings
  - Mutual ex ante information on questions/topics raised in the respective meetings of both groups

Novelties 3

- Expect requests from the Board
    - Potential topics
        - Managing disease areas
        - Affiliated partners and their real life integration

Requests

- Financial support
  - Funding of the common core structure (common pot funding)
  - Financial support on the national level
  - Third party funding

Requests

- Working groups
  - Q: “Are we on the same page?”
- Generally yes
- Room for improvement
  - Participation of MS (reigniting the fire)
  - Composition of WGs
    - Inclusion of further experts with coopted status
      - Ad hoc basis for specific questions/tasks
      - Long-time

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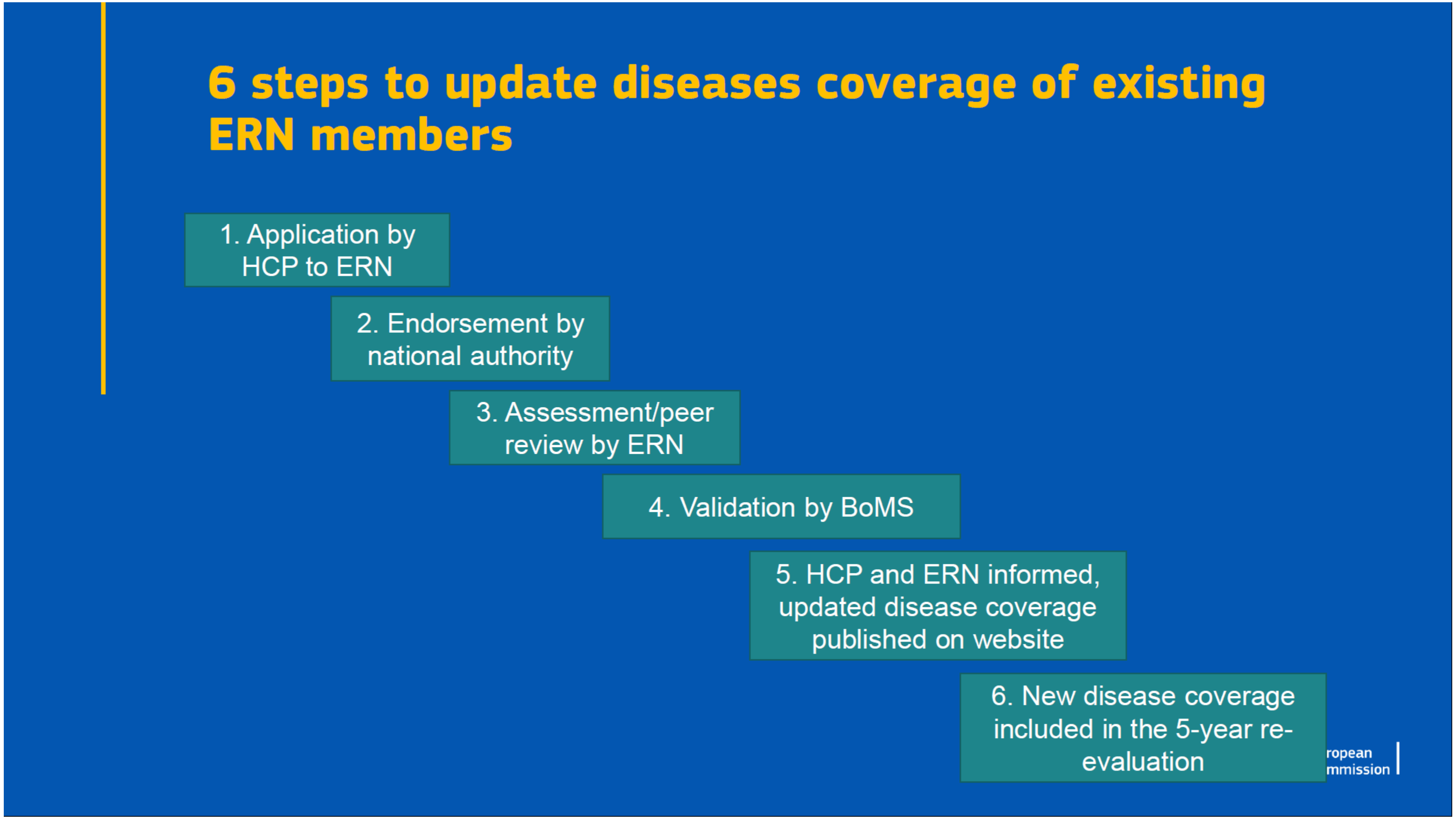
Requests

- ERN registries
  - Technical, legal, ethical challenges
  - BoMS needs more time for reflection

- Monitoring
  - Process “lean and mean”
  - A task for the monitoring working group

- Process for new affiliated partners
  - Window for new AF after approval of new full members (Q4 2021)
  - Timeline needs to be discussed and defined

Further results



6 steps to update diseases coverage of existing ERN members

1. Application by HCP to ERN
2. Endorsement by national authority
3. Assessment/peer review by ERN
4. Validation by BoMS
5. HCP and ERN informed, updated disease coverage published on website
6. New disease coverage included in the 5-year re-evaluation

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Further results

- Managing disease areas within existing networks
  - Discussion about step 2 of the process
  - Solution:
    - HCP has to inform the national health authority about its intention (obligatory step)
    - Explicit / tacit agreement or explicit rejection by the national health authority
    - Adoption by written procedure

Further results

- Working group on integration
  - Document "Reflections on potential Joint Action on Integration of ERNs into national healthcare systems – potential work areas" adopted by the Board
  - "Document of intention"
  - No call for a JA on this topic in 2021

Questions

- How to increase active participation of Member States representatives in the working group?
- How to ensure more legal expertise in the working group?
- Is the BoMS willing to reconsider point 5 of its 2019 Statement on industry, especially with regard to patient registries? (Point 5 of the 2019 Statement: "*There should be no funding from industry directly allocated for management and running of the Network nor for any type of activity relating to the development of diagnostic and clinical practice guidelines or any other clinical decision-supporting tools, development of outcome measures as well as establishing and maintaining patient registries.*")

Further results

- Working group on legal and ethical issues
  - Request to re-consider the 2019 Statement on Industry by the Board

- The board will reflect on that again

Further results

- ERN registries and EU Health Data Space
  - Attention raised to link these two initiatives
  - Further discussion needed how to implement this in practice

Thank YOU

» A big "Thank you" from the Board
for all your enthusiasm
and engagement for
People living with Rare Diseases

Enlargement of the networks

On-going call for new ERN members

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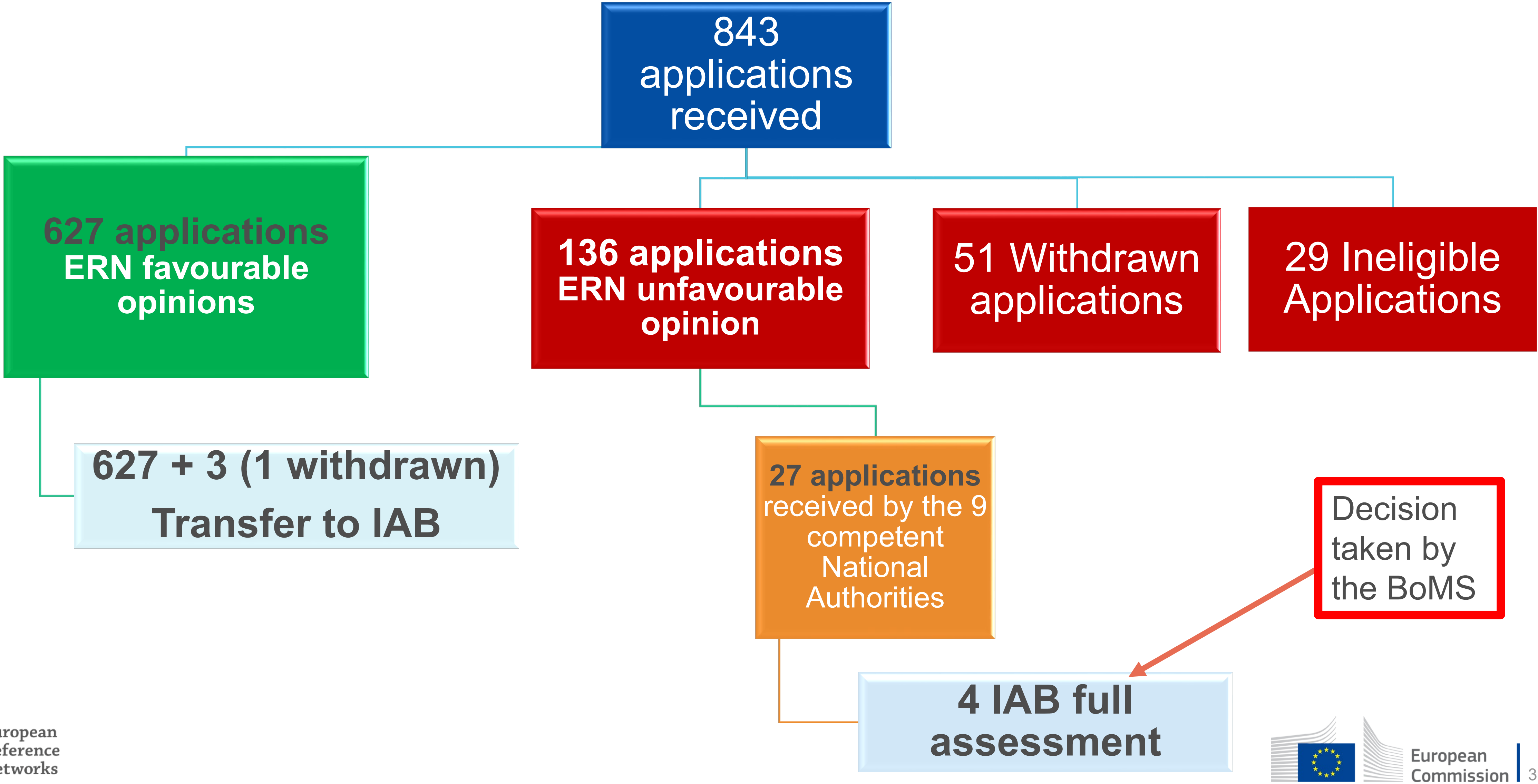
State of play – Calendar (April 2021)

- **IAB review**: March 2021– October 2021
- **Board of the Member States**: November 2021

Current state of play



843 applications received

627 applications ERN favourable opinions

136 applications ERN unfavourable opinion

51 Withdrawn applications

29 Ineligible Applications

627 + 3 (1 withdrawn) Transfer to IAB

27 applications received by the 9 competent National Authorities

4 IAB full assessment

Decision taken by the BoMS

IAB assessment process

19th April – 30th April

12th May-09th June

August 2021

October 2021

Potential new ERNs

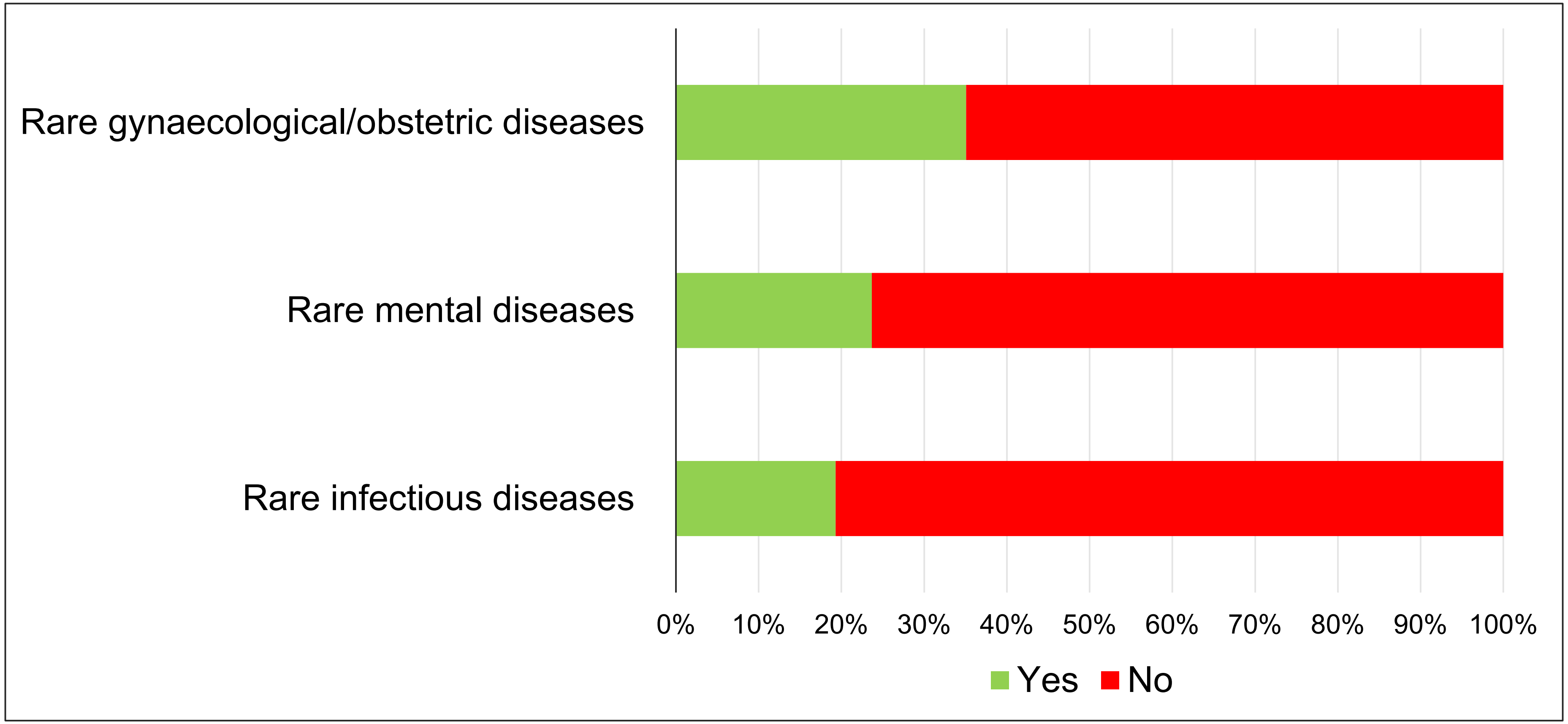
Discussion point

EU survey on potential new ERNs

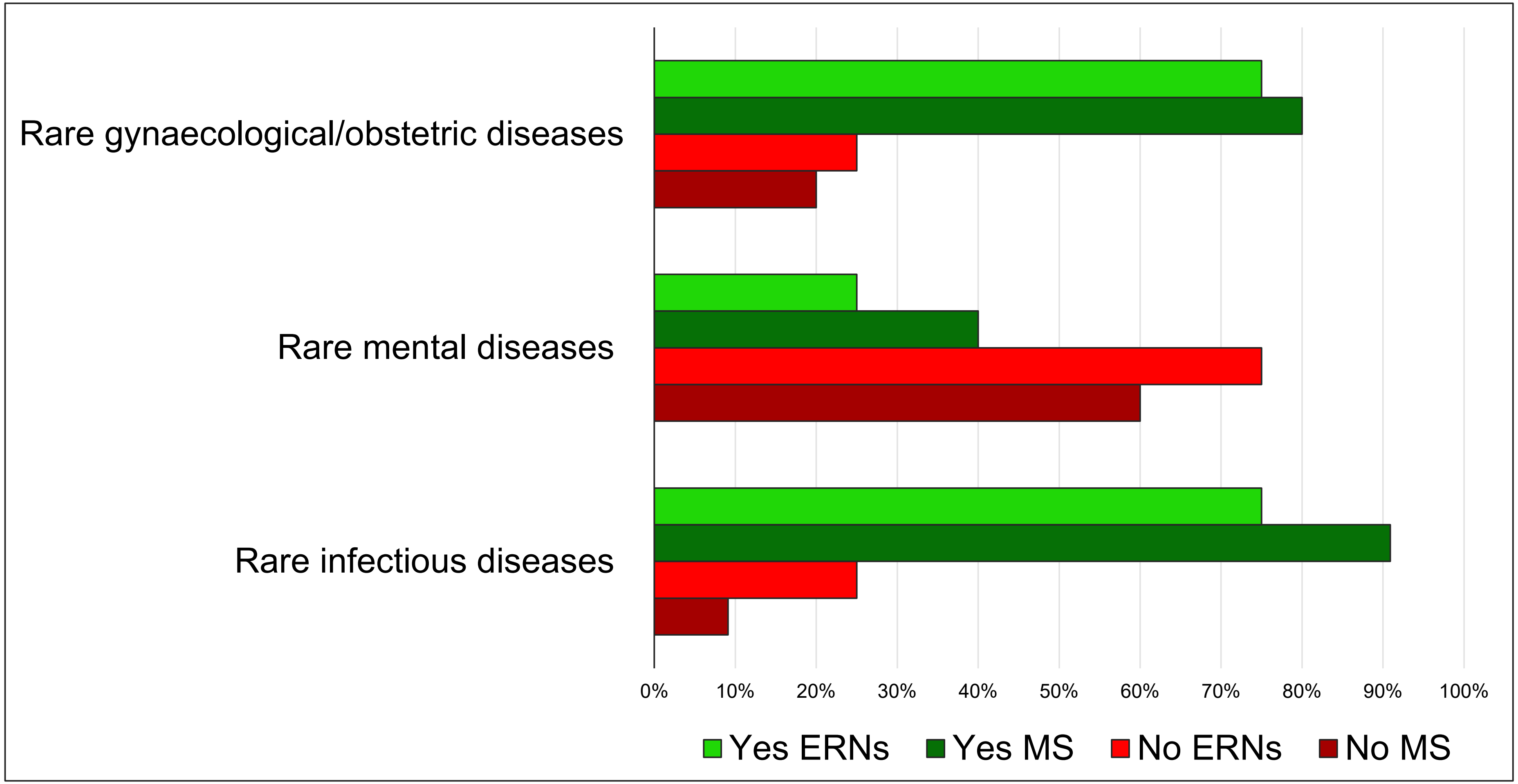
- ➢ ERN on rare gynaecological and obstetric diseases
- ➢ ERN on rare mental disease
- ➢ ERN on rare infectious diseases

- 11 contributions of Member States
- 24 contributions of ERNs (not all ERNs are represented)

Is your ERN already covering this area in any degree?



Do you find the idea of creating a network focused on this area to be relevant?



Rare gynaecological/obstetric diseases

Do you find the idea of creating a network focused on this area to be relevant?

Yes

- Not covered yet and specific expertise is required
- Current unmet need in coverage of developmental aspects of rare diseases

No

- Already covered by other ERNs and transversal working groups
- There could be overlap with other ERNs

Both:

- The diseases need to be clearly defined before making a decision
- Collaboration with other ERNs is important

Rare mental diseases

Do you find the idea of creating a network focused on this area to be relevant?

Yes

- Not covered yet
- Field might benefit from an ERN
- Specific expertise seems required
- May be justified for RD with significant psychiatric symptoms to share knowledge between professionals in different areas

No

- Field is heterogeneous, too complex/wide and too distinct from other ERNs
- Overlap with other ERNs
- Nosology is too vague and moving
- Hard to define rareness in individually varying disease
- Current proposal is unclear
- Cultural differences

Rare infectious diseases

Do you find the idea of creating a network focused on this area to be relevant?

Yes

- Not covered yet
- Important to establish an ERN for (non-)communicable and tropical diseases
- Important to early detect signals and prevent spreading

No

- Infections always affect various organs, which are already covered in other ERNs
- Too much overlap with other ERNs

Both:

- Framework of these diseases need to be further defined
- WHO and ECDC are already highly engaged in this area and need to be involved

Conclusions

- EU survey is non-conclusive
- Participation of all Member States and ERNs is necessary to translate their views into European policies
- More specialised exploratory research is needed

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ERN framework / parallel activities

- Finalise the current assessment of new members of existing ERNs
- AMEQUIS Project: Assessment, Monitoring & Evaluation
- New Framework Contract for Independent Assessment Body
- Action on New ERNs not possible before 2023

Future direct ERN grants

Information point

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Evaluation of the Cross-border healthcare directive

Information point

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Evaluation of Patient Rights' in Cross-border Healthcare

ERN Coordinators' Group – 30 April 2021

Caroline Hager
Team Leader
Cross-Border Healthcare Directive
DG Health & Food Safety

#EUPatientRights #HealthUnion

European Commission

EU legal framework for cross-border healthcare

- **Coordination of social security systems (Regulation No 883/2004)**

- **Directive on the application of patients' rights in cross-border healthcare (Directive 2011/24/EU)**

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Patients’ rights in cross-border healthcare Directive

CJEU jurisprudence from 1998
- Healthcare is a **service**
- Patients can **choose** healthcare provider abroad
- Level of **reimbursement** up to cost of treatment at home
- **Prior authorisation** is acceptable

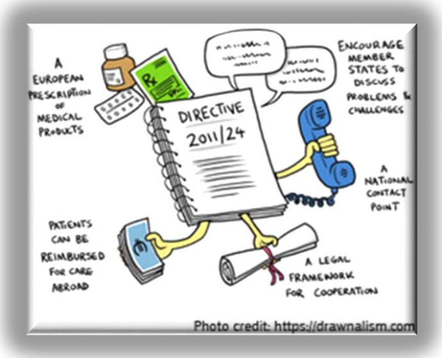


Photo credit: https://drawnalism.com

Harmonized minimum Patients’ Rights

Information to patients

Cooperation between Member States

Kohll and Decker (1998); Ferlini (2000); Geraets-Smits and Peerbooms (2001); Vanbraekel (2001); Inizan (2003); Müller Fauré and Van Riet (2003); Leichtle (2004); Watts (2006); Stamatelaki (2007); Elchinov (2010); Petru (2014) etc.

PROGRESS

- Patients’ rights - choice of healthcare in another EU country
- 200 000 patients take advantage of the Directive to seek healthcare across borders and to be reimbursed for it
- Digitilisation in healthcare – considerable efforts
- High added value of EU actions in the area of rare and complex diseases

CHALLENGES

- few patients aware of their rights to healthcare across borders – clear information to patients
- coherence with the EU social security coordination regulations
- administrative burdens remain

Scope of evaluation

- responsibilities of the Member State of treatment,
- responsibilities of the Member State where the patient is insured provision of information to patients by the National Contact Points on cross-border healthcare,
- administrative procedures for cross-border healthcare,
- recognition of prescriptions issued in other Member States,
- mutual assistance and cooperation in healthcare in the border regions
- **development of the European reference networks and cooperation in rare diseases.**

What do we want to know a decade later?

- to what extent have the Directive’s objectives been met?
- to what extent is the Directive relevant for meeting patient needs in cross-border healthcare?
- how effectively and efficiently does the Directive operate in practice?
- what administrative burdens and barriers do patients still face when seeking healthcare in another Member State and reimbursement thereafter?

What do we want to know a decade later?

- How effective was the Directive to support the diagnosis and treatment of patients with rare and complex diseases?
- How effective was the knowledge sharing?
- What has been the impact of ERNs on research?
- How effectively have Member States and the Commission supported the development of ERNs?

Not included in the evaluation

- Provisions on e-health: will be evaluated separately as part of the preparatory work on the legislative proposal for the creation of a European Health Data Space to be adopted in 2021.
- Cooperation in health technology assessment is a proposal for a Regulation under negotiation (therefore also outside the scope of this evaluation).

But included

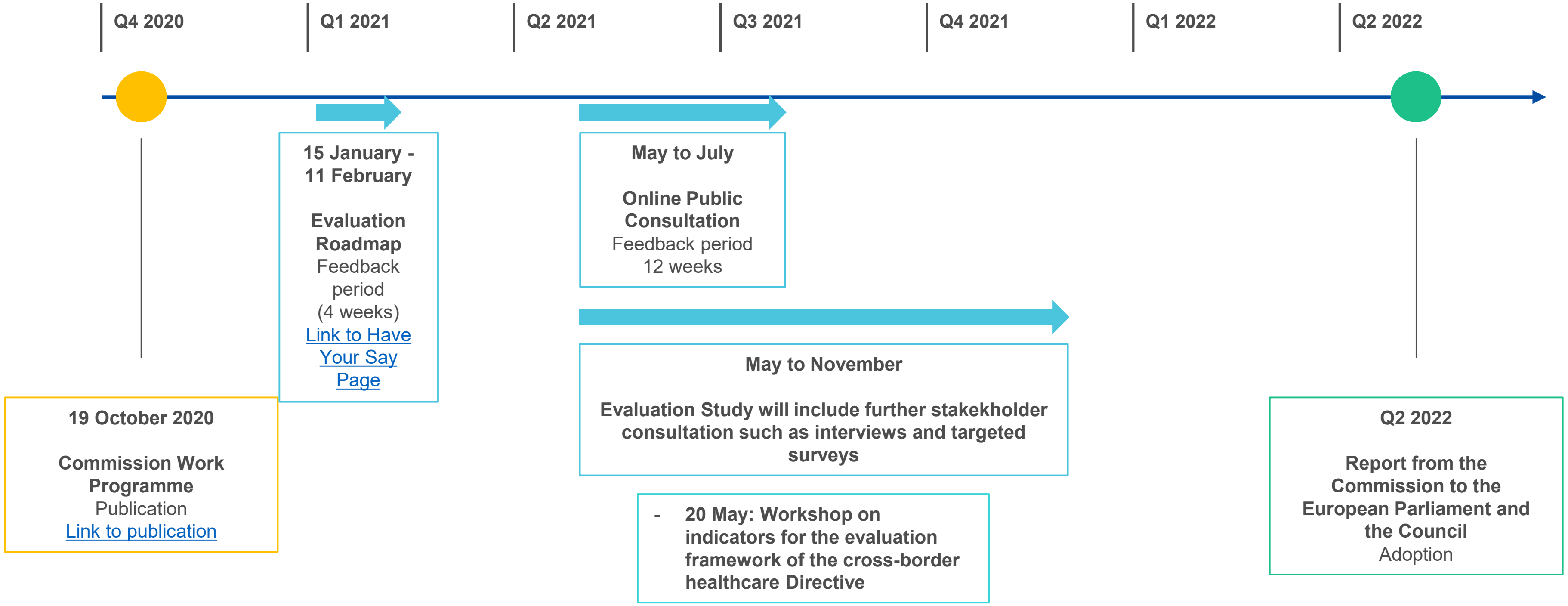
- The Directive’s interaction with other legislation, in particular, with the Regulation (EC) No 883/2004 on the coordination of social security systems

Consultation Strategy

- public consultation 4 May-27 July 2021
- targeted consultation activities (including workshops, interviews) tailored to particular stakeholder groups
- national/regional authorities, National Contact Points for cross-border healthcare, health insurance providers and social security bodies, healthcare providers, health professionals, patient organisations (including organisations representing patients with rare or low prevalence and complex diseases), patient ombudsmen, audit bodies, trade unions, members of the European Reference Network (ERN) Board of the Member States and ERN coordinators.

CBHC Directive Evaluation

Overview of opportunities for stakeholders’ feedback



Q4 2020 | Q1 2021 | Q2 2021 | Q3 2021 | Q4 2021 | Q1 2022 | Q2 2022

19 October 2020

Commission Work Programme
Publication
[Link to publication]

15 January - 11 February

Evaluation Roadmap
Feedback period
(4 weeks)
[Link to Have Your Say Page]

May to July

Online Public Consultation
Feedback period
12 weeks

May to November

Evaluation Study will include further stakeholder consultation such as interviews and targeted surveys

- **20 May: Workshop on indicators for the evaluation framework of the cross-border healthcare Directive**

Q2 2022

Report from the Commission to the European Parliament and the Council
Adoption

You can find further information on the evaluation and the consultation process [here].

This timeline is indicative and for information purposes only. It may be subject to changes.

Coffee break

11:40-11:55

European Reference Networks

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Managing disease areas within existing Networks

Discussion point

European Reference Networks

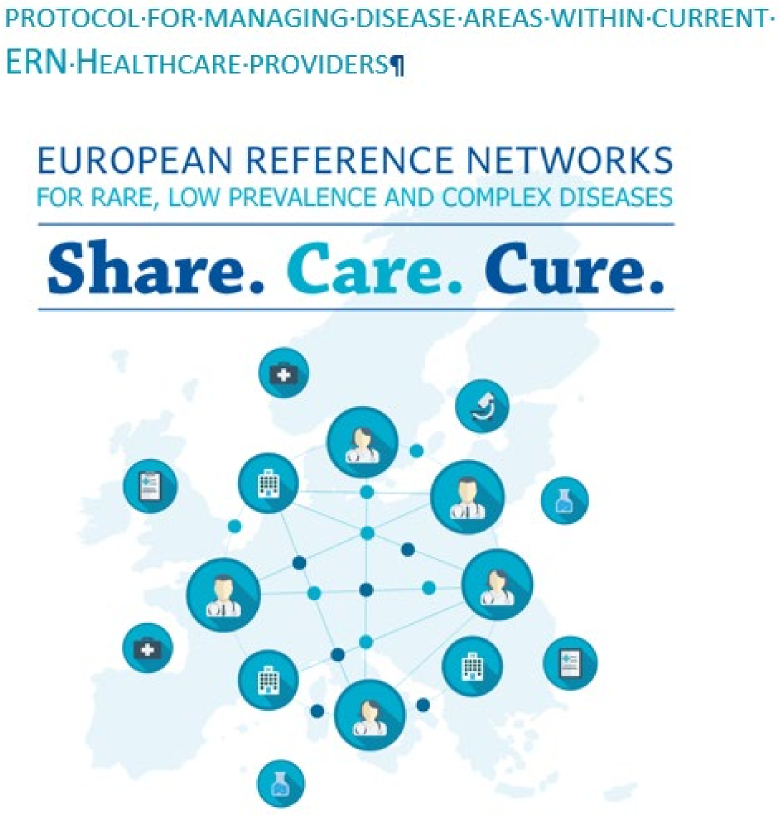
European Commission

Background

- New disease areas added to the coverage of several ERNs in 2019
- Commission included those new diseases under the eligible areas of expertise for the new applicants under the 2019 call
- Need to establish a dynamic procedure to update the disease coverage of the existing ERN members and to fill the gaps**:**
    - to expand into the initial disease areas of their ERNs for which these members did not initially apply in 2016.
    - to expand into the new disease areas included by their ERNs in 2019.

Protocol for managing diseases areas within Current HCP

- First draft developed by drafting group of ERN Coordinators and the Commission
- Presented to BoMS at November 2020 meeting
- Written comments received from BoMS and included in new draft for BoMS April 2021 meeting
- Objective: lighter dynamic procedure for continuous updating of disease coverage of ERN members



PROTOCOL·FOR·MANAGING·DISEASE·AREAS·WITHIN·CURRENT·
ERN·HEALTHCARE·PROVIDERS¶

6 steps to update diseases coverage of existing ERN members

1. Application by HCP to ERN
2. Endorsement by national authority
3. Assessment/peer review by ERN
4. Validation by BoMS
5. HCP and ERN informed, updated disease coverage published on website
6. New disease coverage included in the 5-year re-evaluation

Proposed procedure

- Annual cycle
- No need to assess general criteria, focus on assessment of specific criteria related to the specific disease concerned
- Flexibility for ERNs to arrange the assessment process by, for example:
  - smaller assessment committee of the ERN
  - or evaluation group consults ERN board
  - or the whole ERN board participating in assessment

Review of legislation on orphan medicinal products

Information point

Revision of the EU Orphan Regulation

ERN Coordinators Group meeting, 30 April 2021

Kaja Kantorska

DG SANTE

1. Timeline

- August 2020: Evaluation of the orphan legislation
- November 2020: Impact Assessment Roadmap
- 1Q - 3Q 2021: Impact Assessment study
- Legislative proposal: 1Q 2022

Summary of problems

- Insufficient development in areas of greatest unmet medical needs
  - ➢ 95 % rare diseases no treatment option
  - ➢ ‘One-size-fits-all’ incentives and rewards <-> unmet needs
- Availability and *accessibility* varies across MS
  - No link between incentive and placing on market (orphans)
  - Limited generic competition after expiry of exclusivity periods
- Scientific and technological developments cannot be fully exploited
  - ➢ Instruments not adequate for advances in science: biomarkers and personalised medicine
- Certain procedures inefficient and burdensome

Objectives of the revision:

- To foster research and development of medicines for rare diseases in areas of unmet need and in better alignment with patient needs
- To ensure availability and timely access of patients to orphan medicines
- To ensure legislation to be fit to embrace technological and scientific advances
- To provide effective and efficient procedures, for assessment and authorisation

Policy options

Common elements to all options

- Boost developments in unmet needs: Criteria for unmet medical need in rare diseases (PRIME like scheme for regulatory support/research funding for academia and SMEs)
- Improve accessibility: Faster generic competition/possibility transfer to another company/temporal validity designation for timely development/cumulative numbers people affected
- Adapt to scientific developments: Definition of orphan condition (no orphan designation for subsets of common diseases)
- Procedures more efficient: discard ‘insufficient return on investment’ criterion/better coordination between Agency committees

Policy options

- **Option 1 - change in the incentives**
  - ➢ Prevalence threshold 5 in 10 000 will remain same
  - ➢ Main incentive: market exclusivity (max. 10 years)
    - o Initial length depends on type of development (innovative vs. repurposed)
    - o Extension on basis criteria insufficient return investment/availability in MS/prevalence threshold

- **Option 2 – change in the scope**
  - Changes to current criteria for designation
    - ➢ Changes to current threshold of 5 in 10 000
    - ➢ Different criterion (incidence?) for specific rare diseases

Policy options

- **Option 3 – Options 1-2 + novel incentives for UMN**
  - ➢ Market exclusivity will remain standard incentive (fixed and variable elements)
  - ➢ Alternative (novel) incentive for unmet need in rare diseases and rare paediatric diseases (e.g. extension of regulatory incentive, transferable voucher/SPC)
  - ➢ Complement or replace market exclusivity (assessment on impact availability/access/competition + conditions/limitations)

- **Option 4 – Options 1-3 but ME and novel incentives only for UMN**
  - ➢ Market exclusivity not an incentive for all medicines for rare diseases
  - ➢ Market exclusivity or novel incentive for unmet need in rare diseases and rare paediatric diseases

Next steps

- Beginning May 2021: Open Public consultation for 12 weeks
- Later in May 2021: targeted surveys and interviews

Thank you

Update on AMEQUIS project

Information point

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AMEQUIS project

An integrated Assessment, Monitoring, Evaluation and Quality Improvement System (AMEQUIS) for the European Reference Networks

to improve the ERN system based on a continuous quality improvement system approach for:

- **the assessment** of the new ERN or HCP application;
- **the monitoring** of the activities and the deliverables developed by ERNs and their members;
- **the evaluation** of the ERNs and their members;

Expected outcomes and impact

- Integrating an evaluation system that measures the ERN impact
- Increasing accountability of the ERNs
- Improving patient-centred care and clinical outcomes
- Opportunities for benchmarking of common indicators
- Instituting a harmonized application process of the ERNs
- Reinforcing ERNs with a robust quality improvement process
- Opportunities for improving efficiency of the ERN

WHAT has been done? (January-April 2021)

- **Literature review:** assessment, evaluation and monitoring
- **Collection of input** from the Independent Assessment Bodies
- **Stakeholders' consultation:** assessment, evaluation, and monitoring
  - Stakeholder Awareness Meeting (15th February 2021)
  - Stakeholders Survey (15th March - 14th April)
  - Stakeholder's Conference (12th-13th April)

NEXT STEPS

- Conclusions from 1st Conference → Circulation as slides set for comments, May 2021.
- 2nd Stakeholder Conference → November 2021.
- End of project: December 2021.

Potential involvement of ERN coordinators in defining future projects

Discussion point

European Reference Networks

European Commission

ERN Working Groups and their reporting

General issues

Discussion point

European Reference Networks

European Commission

Lunch break

13:00-14:00

Report from Working group on ERN integration

ERN BoMS meeting

Working Group on ERN Integration

POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS

2021-04-29

Timeline

EU4Health Regulation

https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A52020PC0405

Annual Work Programme (2022)

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

Drafting of the document: 2020 Q2-3.

Meetings: 2020-11-25; 2021-01-21; 2021-04-14.

Discussions/adoption in the **BoMS** (2021-04-29) and ERN CG (2021-04-30).

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS:

POTENTIAL WORK AREAS

- **General support of BoMS and ERN CG members:**

The governance structure of ERNs that enables creating, pooling and sharing knowledge as well as the discussion on rare or low prevalence complex diseases is in place. To reap all benefits for patients, effective mechanisms to connect ERNs' with national health systems need to be developed. A potential way forward in this area is through a Joint Action (JA) supported by the European Commission under the EU4Health Programme.

- **The main topics** identified in the 2019 ERN Board of the Member States **Statement on integration** should be taken as a starting point.

Methodology

Taking into account **diversity and autonomy** of national health systems (NHS), varying degrees of experience and already existing best practice examples in tackling common issues, general applicable methods for the implementation of the tasks listed below would be:

- collection of data on the **state-of-the-art**,
- sharing of **best practice examples**, and
- development of **models, recommendations or general principles** that are adjusted and applied according to legal/ organizational situation in every NHS.

Exact methodologies applied to every task will be defined later on.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

General provisions

- Aims and tasks are mainly **targeted at MS and national health systems** (NHS) as those who are responsible for implementation with the aim to reach consensus among MS for various processes in the NHS that are

1) of crucial importance for ERNs' functioning and sustainability and
2) of crucial importance for MS to reap ERNs benefits.

- For the identified tasks we still do not have exact and optimal to everyone answers – hence the need for JA;

- For many tasks, there will not be just one answer – **flexibility** is a key when we talk about so diverse and autonomous NHS and ERNs, hence, we talk about model/ several models or general principles that are adjusted and applied according to legal/ organizational situation in every NHS.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

I. Governance framework for ERNs integration in the national health systems

- Develop a proposal for **national governance models and practices** for rare and complex disease centres and pathways that are fully interoperable with ERNs and take into account differences of national health systems (NHS). The main aims are to organize all national HCPs providing care to patients with rare and complex diseases according to the principles of proper balance between HCP competitiveness and centralization of resources to ensure high quality, effectiveness and safety of services (e.g., through the establishment of national networks or national reference hubs) and to reduce administrative and coordination burden for ERNs.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

I. Governance framework for ERNs integration in the national health systems

- Develop recommendations for **national data governance models and practices** to ensure interoperability between national and local data structures (including e-health systems, national and regional registries, hospital information systems) and ERN data structures (including ERN registries and Continuous Patient Management System CPMS), taking fully into account the ongoing work on the European Health Data Space, Joint Action Towards the European Health Data Space and the preparations of the legislative proposal to be adopted by the Commission in 2021. Data governance models and practices should take into account FAIR principles, rare disease codification and ontology systems.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

I. Governance framework for ERNs integration in the national health systems

- ❑ Develop a proposal for an **EU-wide monitoring framework for a NHS-level monitoring** of indicators for rare and complex diseases that are relevant to ERN integration. The monitoring framework should include indicators specific for rare and complex diseases, data collection methods, timelines and regularity, definition of roles and responsibilities, and fully take into account the ongoing work of continuous ERN monitoring system and AMEQUIS project.
- ❑ Develop a proposal for **national quality assurance models** for rare and complex diseases with the aim to ensure <u>coherent quality and safety standards in centers and pathways</u> for rare and complex diseases and <u>in the ERNs</u> and taking into account <u>differences of NHS</u>.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

II. Patient care pathways

- ❑ Develop a proposal for the **organization of national care pathways** for rare and complex diseases that <u>interface with ERNs </u>and take into account <u>differences of NHS</u>. The recommendations shall establish the link between ERN model-methodologies and operational national care pathways, including processes to adopt and/or adapt <u>evidence-based resources </u>(as Clinical Practice Guidelines) developed by ERNs.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

III. Referral systems to the ERNs

- ❑ Develop a proposal for the **organization of referral systems** to the ERNs, including national criteria, good practice and guidelines for CPMS usage, incorporation of CPMS advice into patients' care, possibility of "CPMS-first" strategy for cross-border healthcare services, taking into account legal and organizational differences of NHS.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

IV. Education and awareness raising on ERNs at national level

- Prepare a blueprint for a **national dissemination/ communication strategy** on the ERNs and **patient empowerment** targeted at multistakeholder communities at national level.
- In cooperation with the ERN Knowledge Generation Working Group, develop a proposal for **national strategies for healthcare workforce development** that are aligned with the educational and training programs developed by ERNs, take into account the needs of different stakeholders and long-term healthcare workforce sustainability.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

V. Sustainability of the ERN model at HCP level

- Identify **HCP-specific needs and benefits** to enable efficient participation in ERN activities and propose mechanisms to address them. Develop overview of good practices of mechanisms to provide support to the HCPs that participate in the ERNs at the national level.
- Define **indicators linked to the provision of funding** for ERNs and ERN integration in order to (i) demonstrate the benefits of the ERN model for patients and NHS in relation to the funding provided and (ii) thus escalate political leadership and ownership of the ERN system at national level. Include national funding institutions in this work.

REFLECTIONS ON POTENTIAL JOINT ACTION ON INTEGRATION OF ERNS INTO NATIONAL HEALTHCARE SYSTEMS: POTENTIAL WORK AREAS

V. Sustainability of the ERN model at HCP level

- ❑ Develop guidance and recommendations on **CPMS reimbursement models**.
- ❑ Based on this overview define a set of specific **recommendations** on how the Member States should support their HCPs and Affiliated partners and a mechanism for monitoring the progress (implementation of the recommendations).

Warm acknowledgments for all your time, suggestions, discussions, critics –

time for questions!

Report from
Working Group on Monitoring

European
Reference
Networks

European
Commission

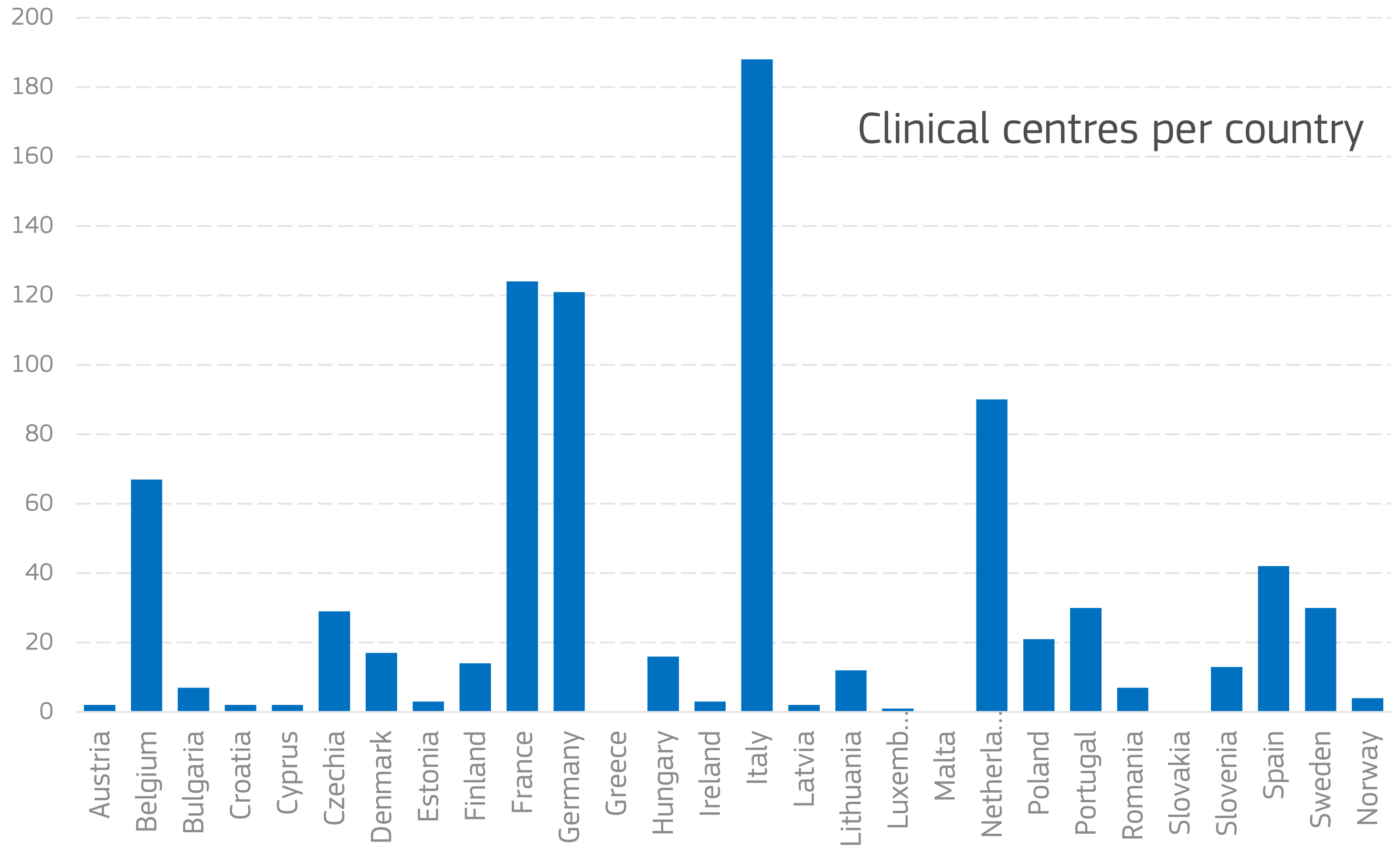
Main actions of the Monitoring WG in 2020

- Intensive activity and collaboration
- Improvement of indicators system, awareness and training
- Four meetings
- Two data collection exercises and two Webinars for all ERNs

Data collections of 2020

- Improved participation, including Affiliated Partners for the first time
- Goal: not to compare ERNs, but to assess the overall performance
- Data to be made public (a selection of indicators)
- Problems:
  - No advancement in other key elements to be monitored (e.g. integration)
  - Limitations due to COVID-19 and Brexit

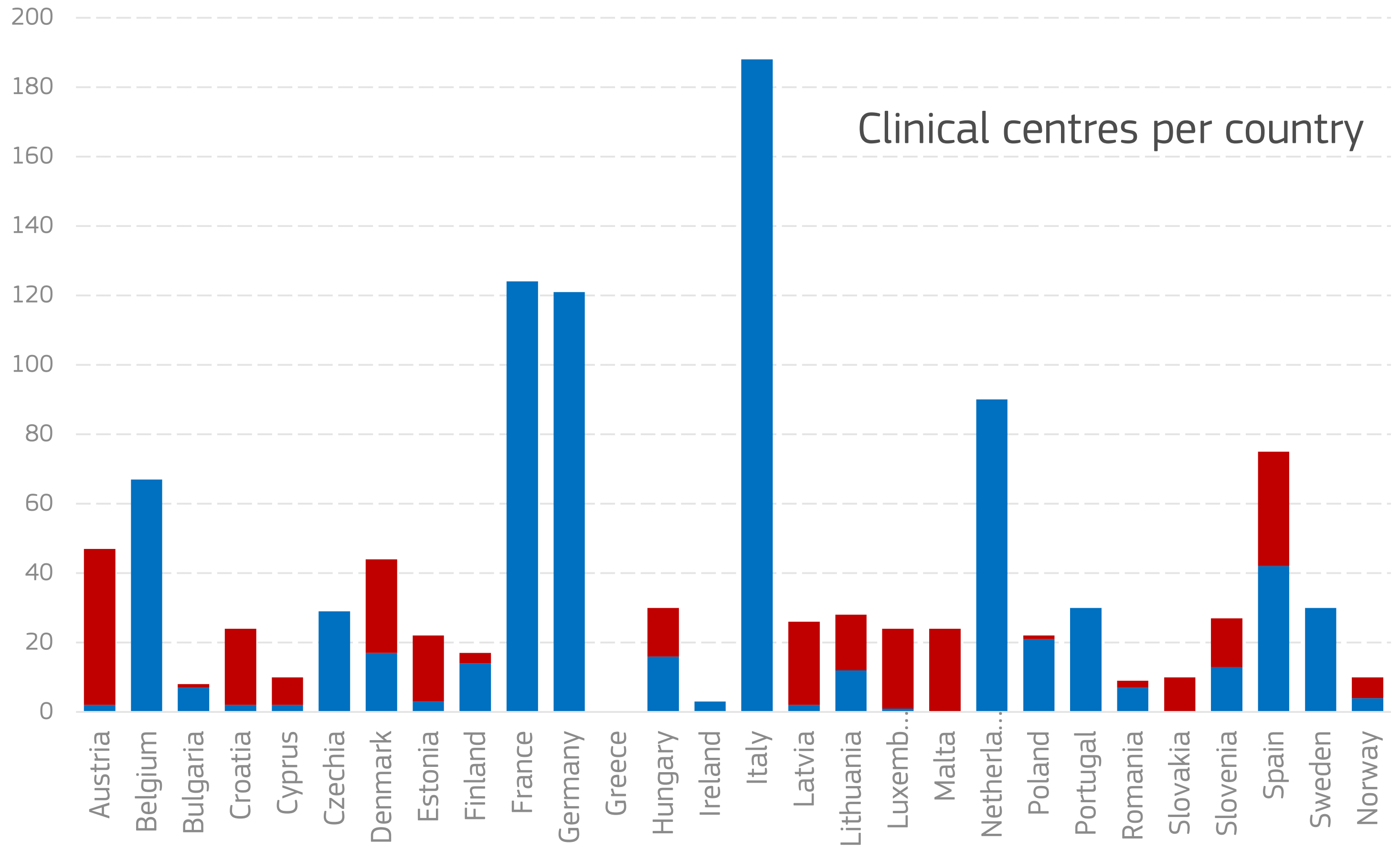
Geographical coverage before affiliated partners



European Reference Networks

European Commission

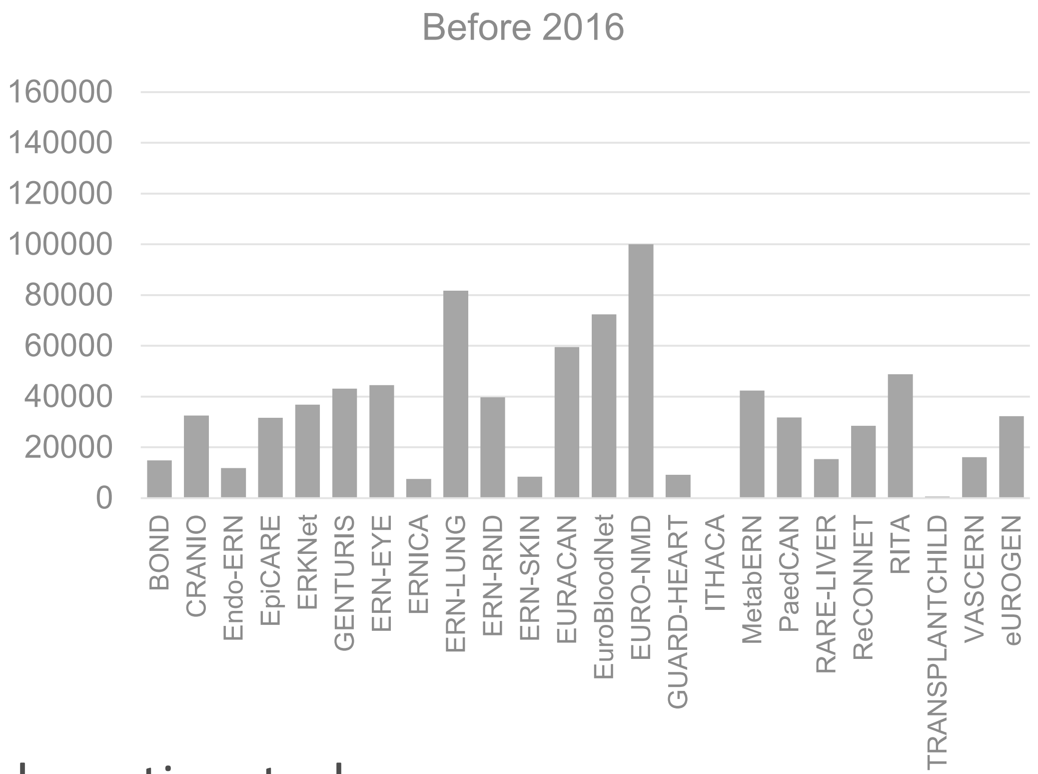
Geographical coverage after affiliated partners



European Reference Networks

European Commission

Patient distribution

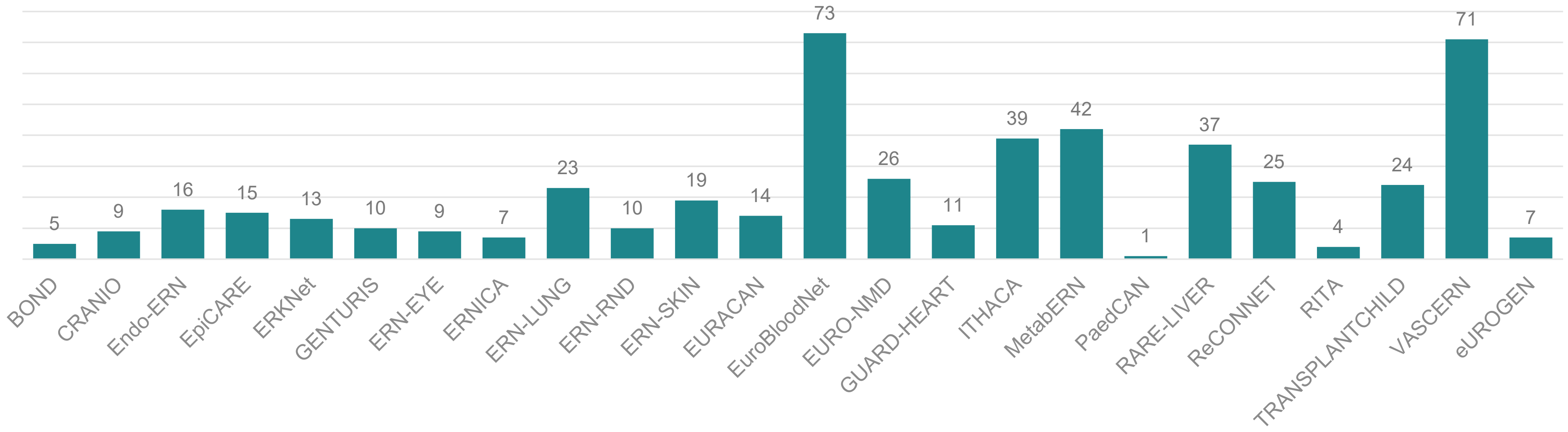


- Total ERN population: 1.67M, probably underestimated
- HCP participation in 2020: 88% (COVID + Brexit)

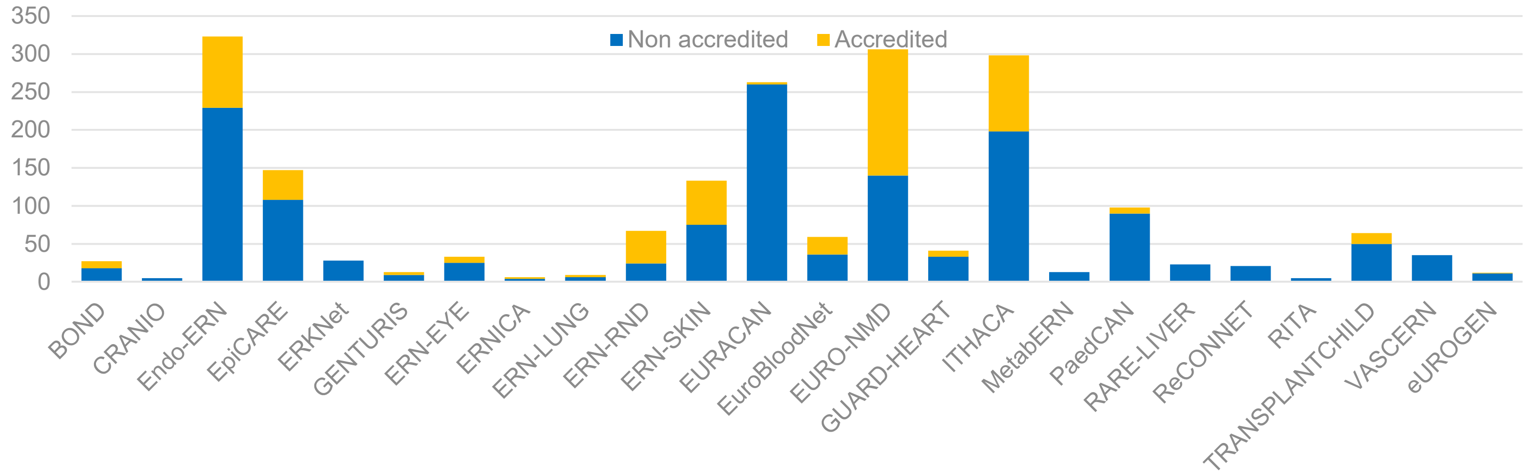
European Reference Networks

European Commission

Patient organisations

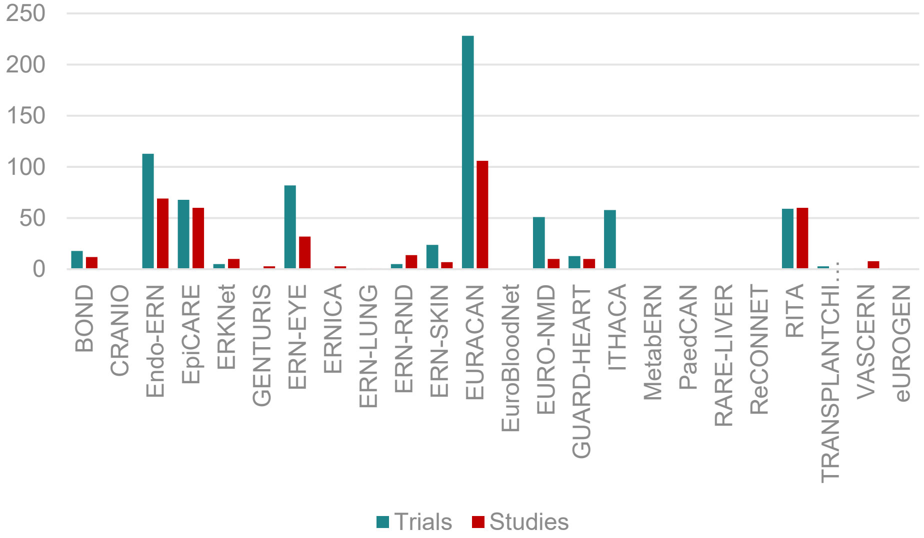


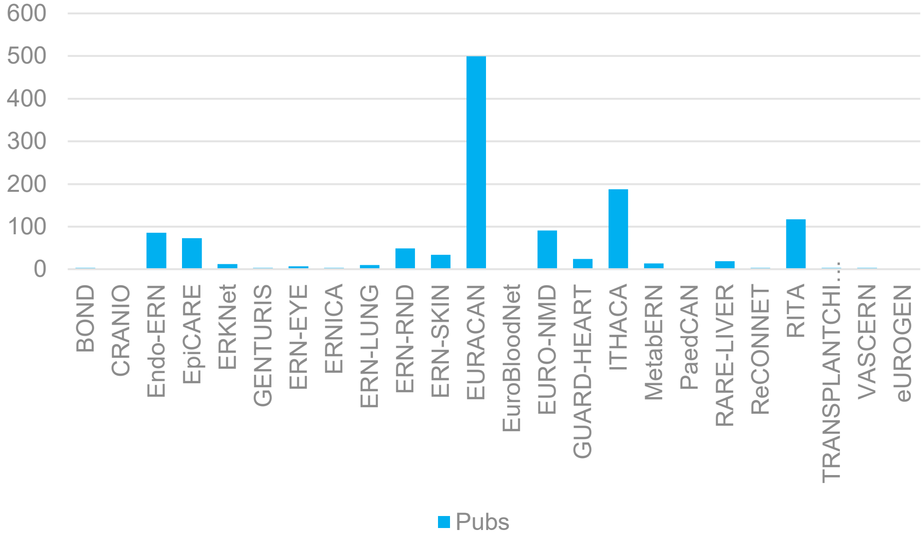
Training activities



- Some networks do not provide enough evidence
- Some double counting is possible
- HCP participation: 92%

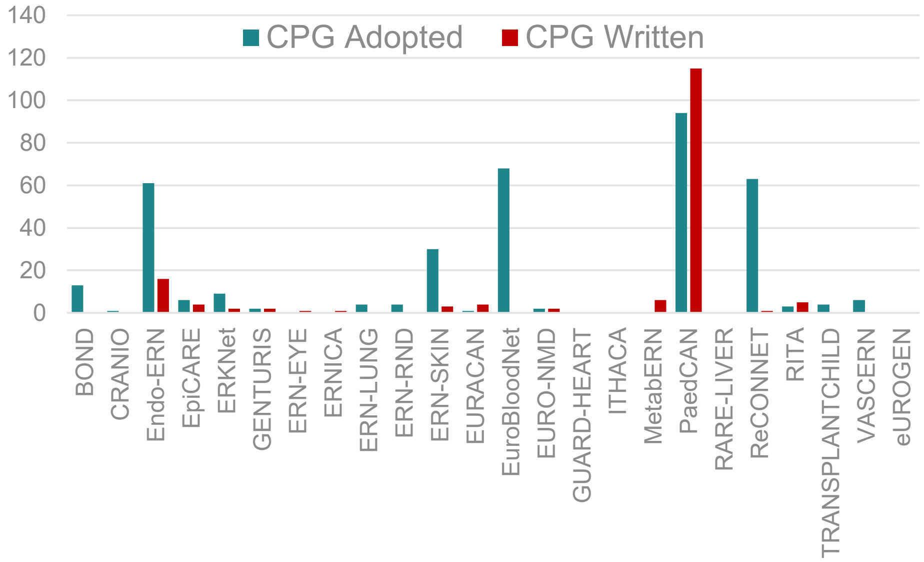
Research activities

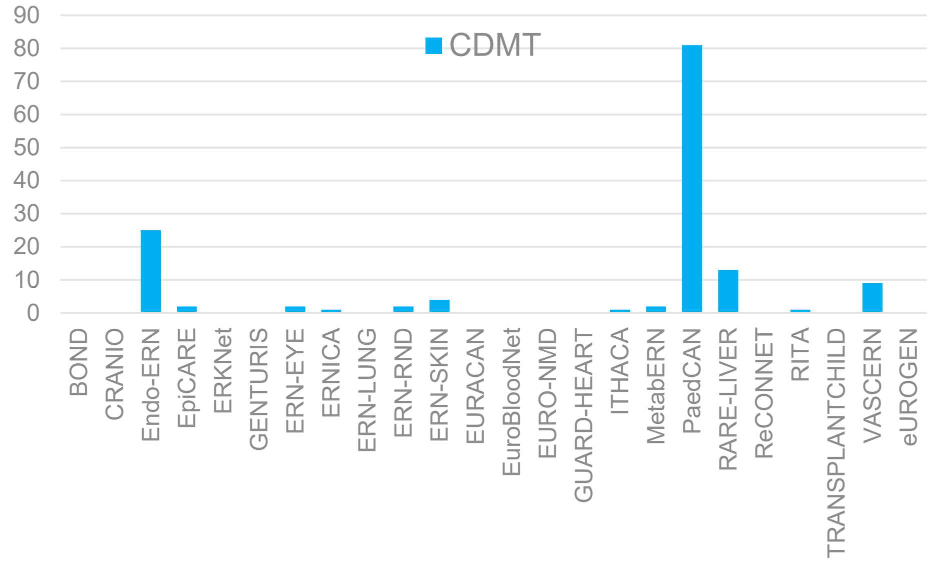




- Often acknowledgement of the ERN is missing, sometimes because activities are funded from other sources
- HCP participation: 92%

CPGs and CDMTs





- Adoption of CPGs is in progress or not yet in place
- Possible counting of activities that are missing ERN acknowledgement
- HCP participation: 89%

Conclusions of 2020

- Estimated ERN population: around 1.7 million (increasing)
- Participation of patient organisations: median 14 (increasing)
- Increasing engagement in research and educational activities
- Number of ERN Website Hits: around 1 million (stable)

Goals for 2021

- Reach 100% participation of HCPs
  - Evaluation of future multiannual grants will be linked to this participation
  - Non-compliance in reporting data should be linked to termination of HCPs
- Supporting evidence for Indicators to be published on the ERNs websites (publications, meetings, CPG, etc.)
- Inclusion in the AMEQUIS system
- Cooperate with the WG on integration:
  - Define concrete elements that could be subject of monitoring
  - Align with the BoMS statement on integration

Next steps

- Clarifications to some indicators/criteria
- Survey on the Data collection process and possible improvements in the current indicators
- Consistency between the reported information and the information uploaded in the websites for validation/proof of the actions
- Future Data collections (including affiliated partners):
    - 1 to 31 October 2021: data from Jan-Jun 2021 (Webinar on 15.09.2021)
    - 1 to 31 March 2022: data from Jan-Dec 2021 (Webinar on 15.02.2022)

Thank you!

Monitoring Working Group members

- Board of Member States
  Ines Palanca (ES), Till Voitglaender (AT)
- eUROGEN (Lead ERN)
  Wout Feitz (Coord), Michelle Battye (PM), Darren Shilhan
- CRANIO
  Irene Mathijssen (Coord)
- Endo-ERN
  Charlotte van Beuzekom (PM)
- ERKNET
  Franz Schaefer (Coord)
- TRANSPLANT-CHILD
  Eduardo Lopez Granados, Jose Jonay Ojeda Feo
- EURACAN
  Muriel Rogasik (PM)
- EuroBloodNet
  Léa Margot (PM), Victoria Gutierrez (PM)
- ITHACA
  Anne Hugon (PM), Kléa Vyshka (PM), Sarra Selatnia (PM)
- RECONNET
  Sara Cannizzo
- VASCERN
  Guillaume Jondeau (Coord), VASCERN team
- DG SANTE
  Joao de Sousa, Louis Byukusenge, Jose Valverde

Report from Working group on knowledge generation

European
Reference
Networks

European
Commission

European
Reference
Networks

Report from the ERN Working Group on Knowledge Generation

Maurizio Scarpa and György Pflieger,
On Behalf of Knowledge Generation ERN WG

EUROPEAN REFERENCE NETWORKS COORDINATORS GROUP (ERN - CG)
30TH OF APRIL 2021

Knowledge Generation ERN Working Group

Topics for the Discussion

- ***Clinical Practice Guidelines (CPG) and Clinical Decision Support Tools***
- ***ERN Exchange Programme 2021-2022***
- ***ERN Academy***

Clinical Practice Guidelines (CPG) and Clinical Decision Support Tools (CDST) - 1

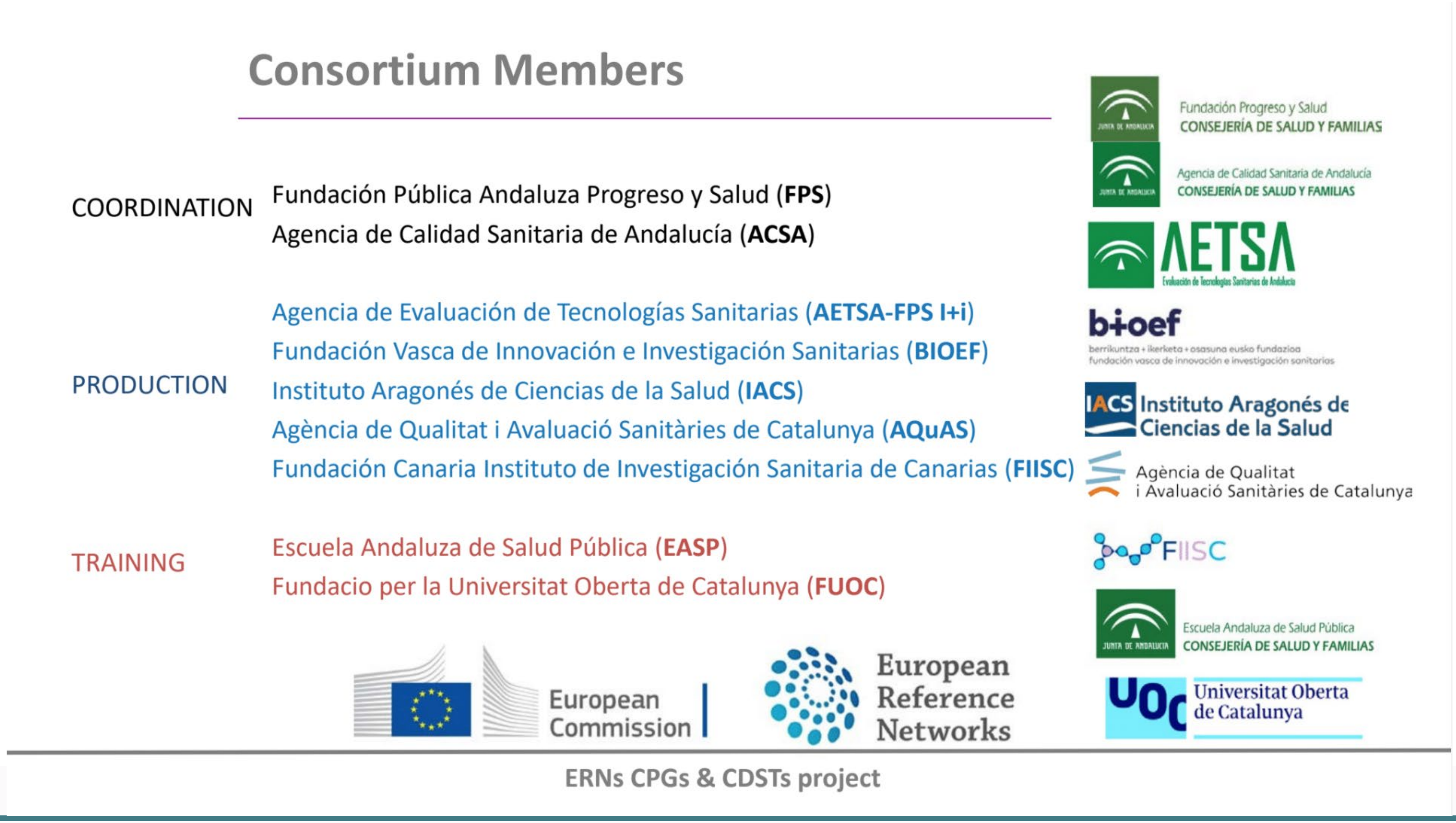
- Project launched in 2020 (consortium: EC + ACSA)
- Clear, straitforward but still flexible organizational structure for development of ERN CPGs and CDSTs – regular survey for modernization (e.g. new diagnostic tools, therapies etc)
- Generally acceptable methodologies for development, appraisal and implementation of CPGs and CDSTs
- Literature review, expert consultation – Methodological Manual and Toolkit (12 Methodological Handbooks); Handbook reviews (AB) – Juan Antonio Blasco
- Training and capacity building
- Training the Trainers March, 2021
- Training program start: 28th April, 2021
- Online learning platform, 4-5 hrs/week up to 2 months, active and flexible (various approaches and tools)

Clinical Practice Guidelines (CPG) and Clinical Decision Support Tools (CDST) - 2

Support for development of 2 new CPGs and CDSTs, appraisal and adaptation of 5 of the already existing ones per each ERN

- EU_Survey to define goals and developmental steps and appraisal was carried out
- Start of CPGs and CDSTs: June 2021 (together with ERN Guidelines Consortium)
- Steps: introduction – prioritization- appraisal – elaboration – evaluation – engagement *(time of each steps should be flexible – both intra-,and interguide)*
- The role of the participants (clinical experts, technical supporting team, patients/organizations) varies from step-to-step

GUIDELINE PROJECT EC



Consortium Members

COORDINATION

Fundación Pública Andaluza Progreso y Salud (**FPS**)
Agencia de Calidad Sanitaria de Andalucía (**ACSA**)

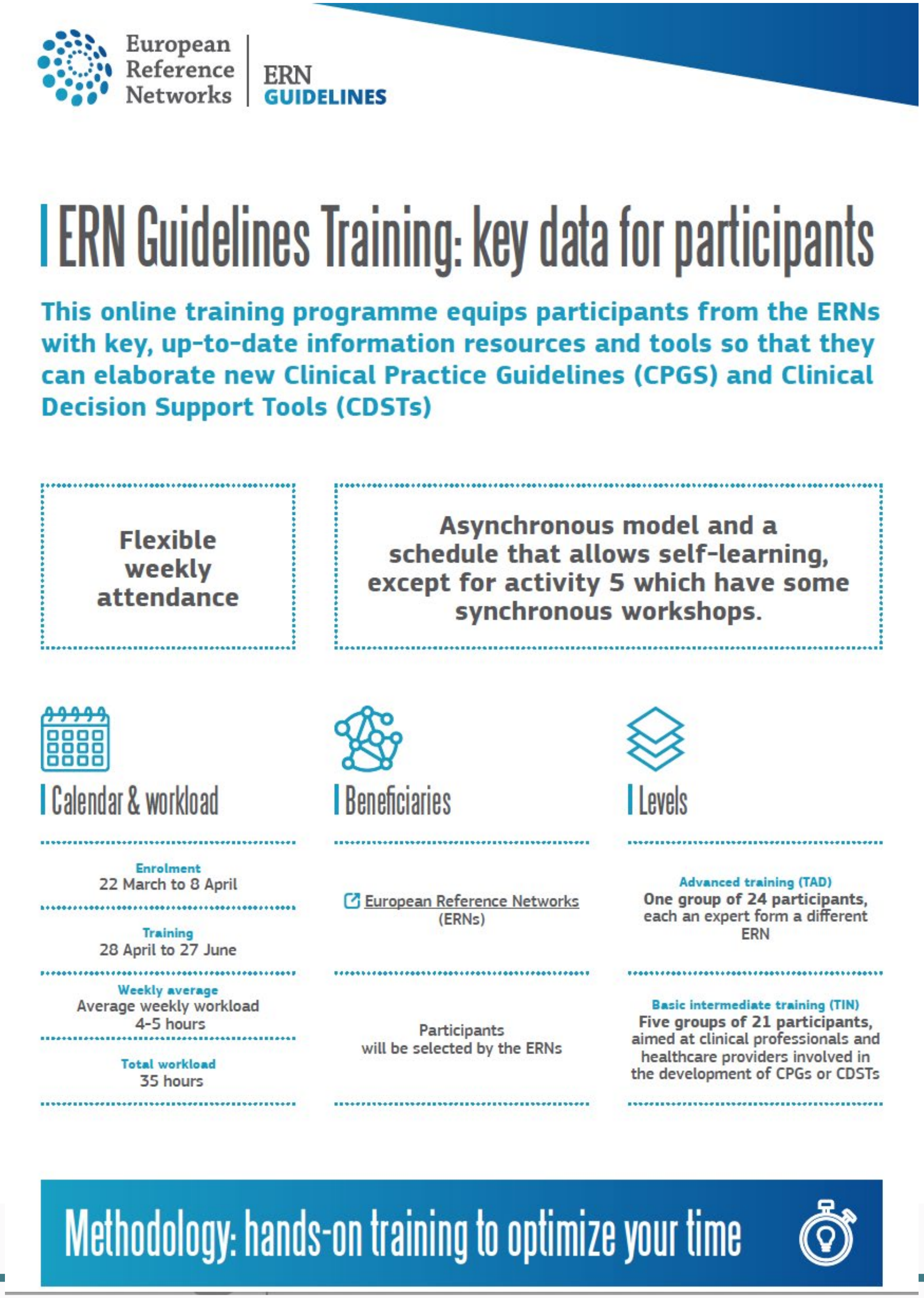
PRODUCTION

Agencia de Evaluación de Tecnologías Sanitarias (**AETSA-FPS I+i**)
Fundación Vasca de Innovación e Investigación Sanitarias (**BIOEF**)
Instituto Aragonés de Ciencias de la Salud (**IACS**)
Agència de Qualitat i Avaluació Sanitàries de Catalunya (**AQuAS**)
Fundación Canaria Instituto de Investigación Sanitaria de Canarias (**FIISC**)

TRAINING

Escuela Andaluza de Salud Pública (**EASP**)
Fundacio per la Universitat Oberta de Catalunya (**FUOC**)

ERNs CPGs & CDSTs project



European Reference Networks | ERN **GUIDELINES**

ERN Guidelines Training: key data for participants

This online training programme equips participants from the ERNs with key, up-to-date information resources and tools so that they can elaborate new Clinical Practice Guidelines (CPGS) and Clinical Decision Support Tools (CDSTs)

Flexible weekly attendance

Asynchronous model and a schedule that allows self-learning, except for activity 5 which have some synchronous workshops.

Calendar & workload

Enrolment
22 March to 8 April

Training
28 April to 27 June

Weekly average
Average weekly workload
4-5 hours

Total workload
35 hours

Beneficiaries

European Reference Networks (ERNs)

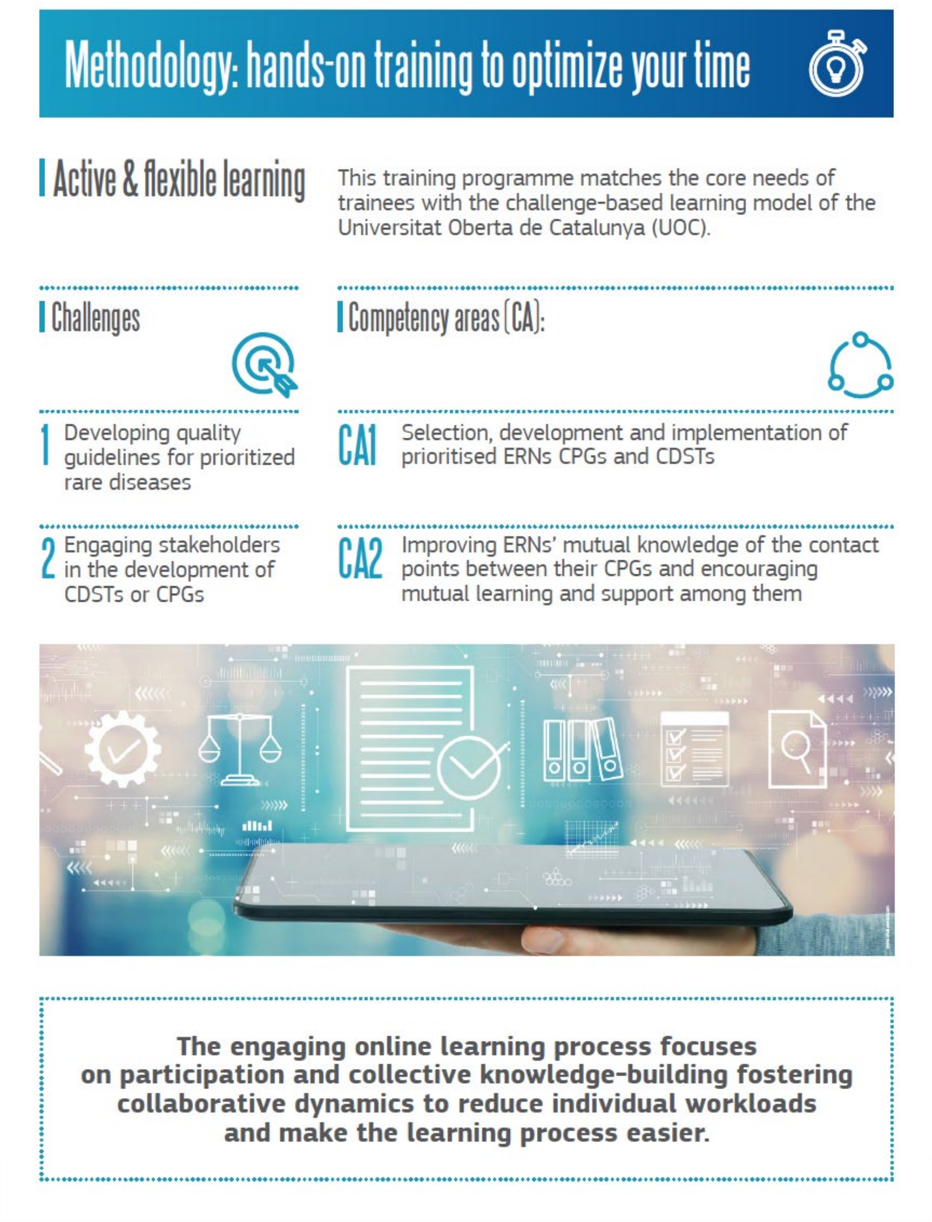
Participants
will be selected by the ERNs

Levels

Advanced training (TAD)
One group of 24 participants, each an expert form a different ERN

Basic intermediate training (TIN)
Five groups of 21 participants, aimed at clinical professionals and healthcare providers involved in the development of CPGs or CDSTs

Methodology: hands-on training to optimize your time



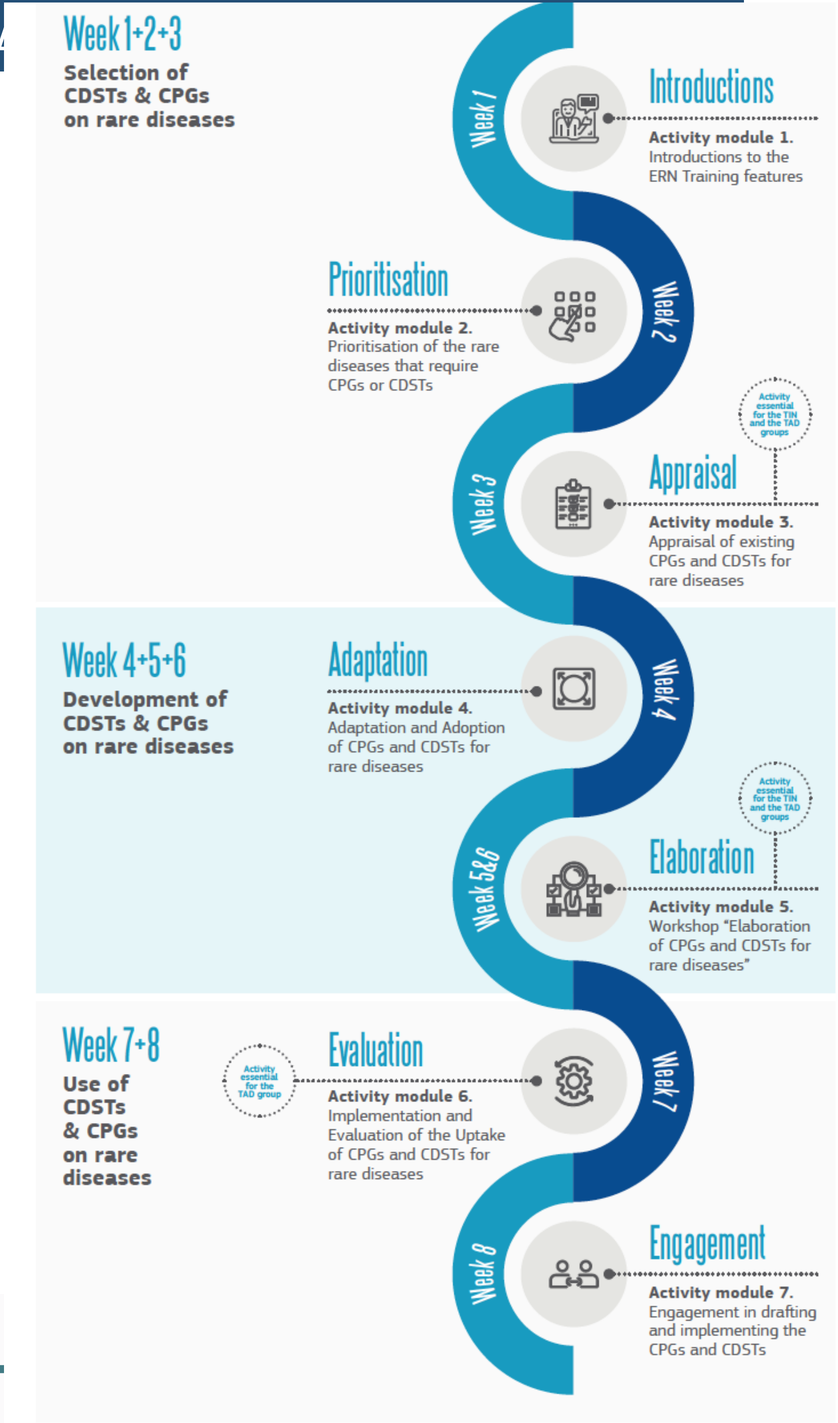
Methodology: hands-on training to optimize your time

Active & flexible learning

This training programme matches the core needs of trainees with the challenge-based learning model of the Universitat Oberta de Catalunya (UOC).

Challenges	Competency areas (CA):
1 Developing quality guidelines for prioritized rare diseases	CA1 Selection, development and implementation of prioritised ERNs CPGs and CDSTs
2 Engaging stakeholders in the development of CDSTs or CPGs	CA2 Improving ERNs' mutual knowledge of the contact points between their CPGs and encouraging mutual learning and support among them

The engaging online learning process focuses on participation and collective knowledge-building fostering collaborative dynamics to reduce individual workloads and make the learning process easier.



Week 1+2+3

Selection of CDSTs & CPGs on rare diseases

Introductions

Activity module 1. Introductions to the ERN Training features

Prioritisation

Activity module 2. Prioritisation of the rare diseases that require CPGs or CDSTs

Appraisal

Activity essential for the TIN and the TAD groups

Activity module 3. Appraisal of existing CPGs and CDSTs for rare diseases

Week 4+5+6

Development of CDSTs & CPGs on rare diseases

Adaptation

Activity module 4. Adaptation and Adoption of CPGs and CDSTs for rare diseases

Elaboration

Activity essential for the TIN and the TAD groups

Activity module 5. Workshop "Elaboration of CPGs and CDSTs for rare diseases"

Week 7+8

Use of CDSTs & CPGs on rare diseases

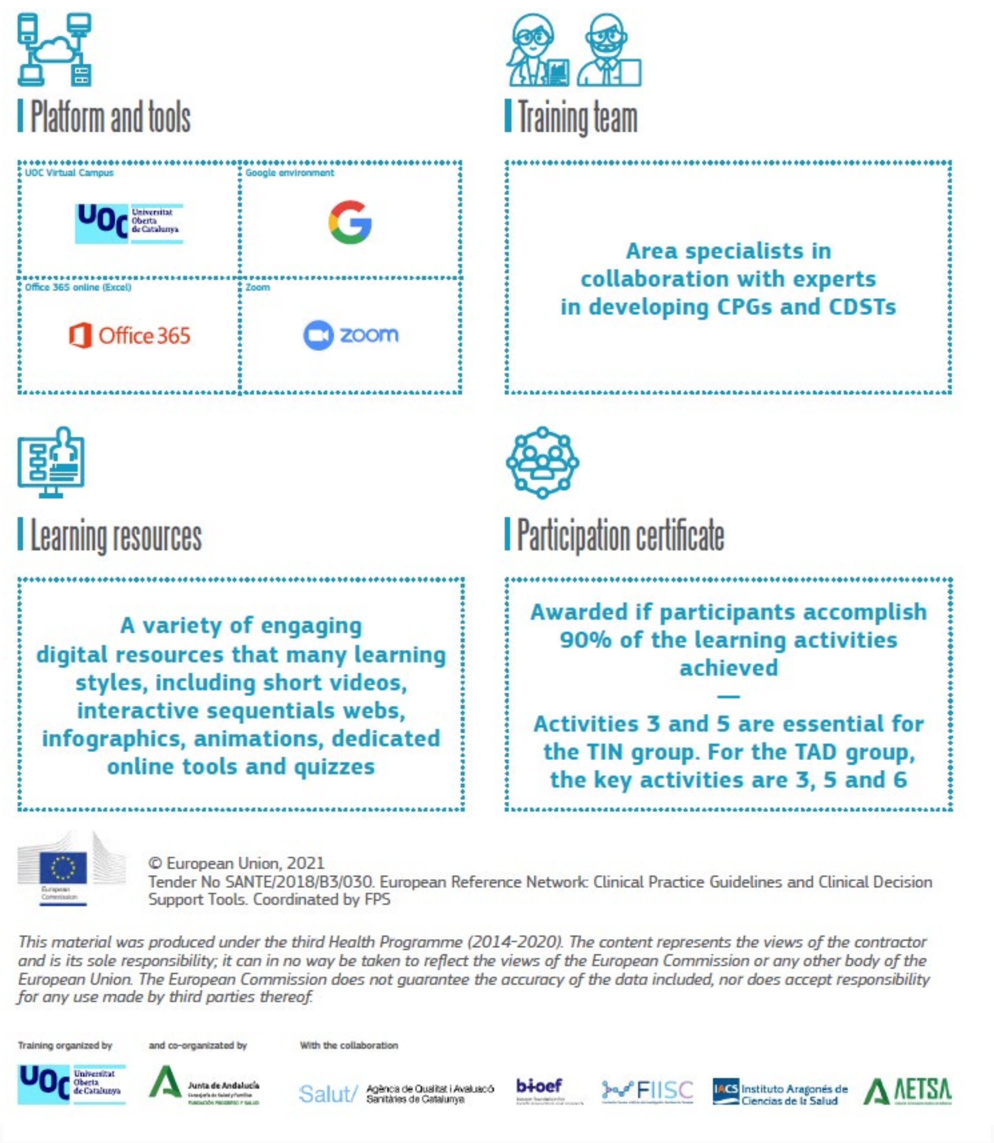
Evaluation

Activity essential for the TAD group

Activity module 6. Implementation and Evaluation of the Uptake of CPGs and CDSTs for rare diseases

Engagement

Activity module 7. Engagement in drafting and implementing the CPGs and CDSTs



Platform and tools

UOC Virtual Campus	Google environment
Office 365 online (Excel)	Zoom

Training team

Area specialists in collaboration with experts in developing CPGs and CDSTs

Learning resources

A variety of engaging digital resources that many learning styles, including short videos, interactive sequentials webs, infographics, animations, dedicated online tools and quizzes

Participation certificate

Awarded if participants accomplish 90% of the learning activities achieved

—

Activities 3 and 5 are essential for the TIN group. For the TAD group, the key activities are 3, 5 and 6

Tender No SANTE/2018/B3/030. European Reference Network: Clinical Practice Guidelines and Clinical Decision Support Tools. Coordinated by FPS

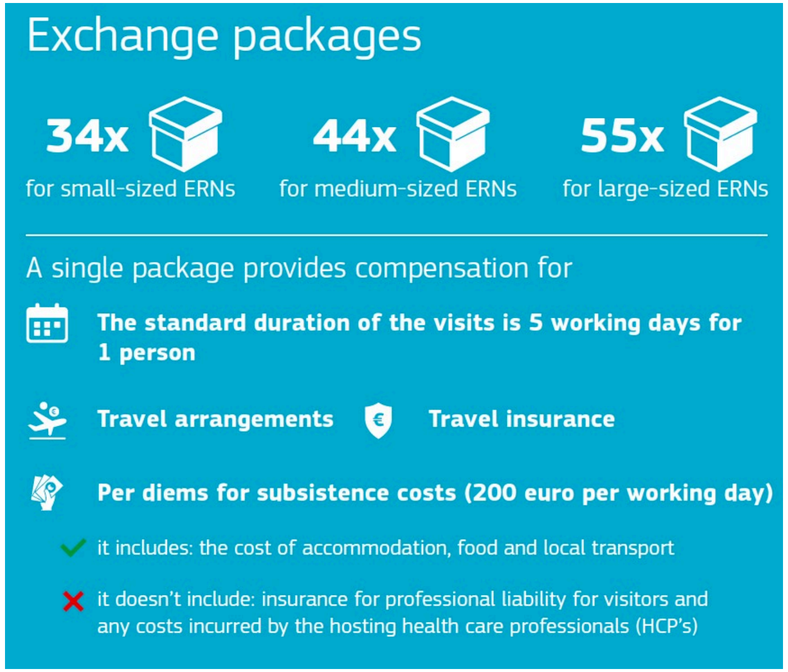
Training organized by

and co-organizated by

With the collaboration

ERN Exchange Programme 2021-2022

- The exchange programme is meant to meet goals and strengthen capacity on the network level.
- The thematic scope includes medical practice and skills but also organisational aspects of a network.
- Each ERN defines the strategic goals and priorities of the exchange programme, according to the specific situation of the network.
- Health professionals working in the ERN centres are eligible for the programme – this includes all disciplines related to the relevant expertise area of the ERN.
- Formally affiliated member HCPs are also eligible for exchange visits.



Exchange packages

34x for small-sized ERNs

44x for medium-sized ERNs

55x for large-sized ERNs

A single package provides compensation for

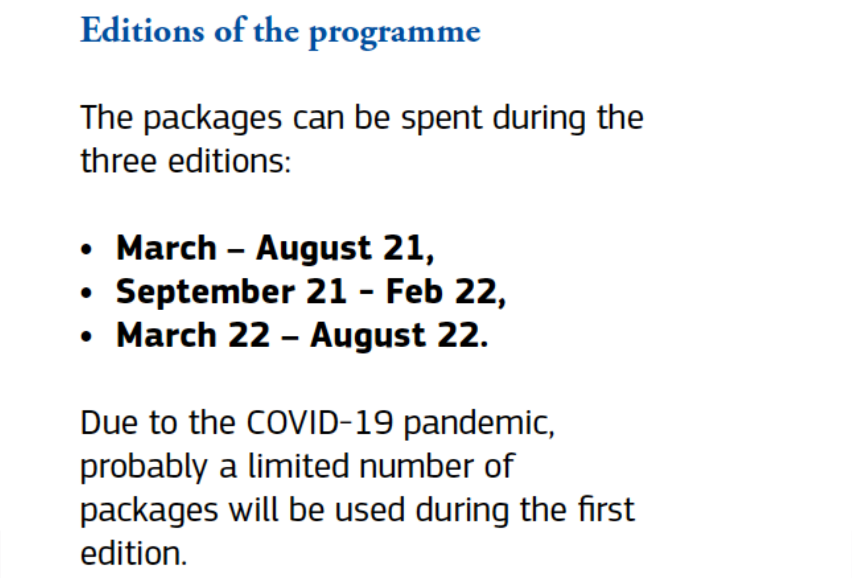
The standard duration of the visits is 5 working days for 1 person

Travel arrangements **Travel insurance**

Per diems for subsistence costs (200 euro per working day)

✓ it includes: the cost of accommodation, food and local transport

✗ it doesn't include: insurance for professional liability for visitors and any costs incurred by the hosting health care professionals (HCP's)



Editions of the programme

The packages can be spent during the three editions:

- **March – August 21,**
- **September 21 - Feb 22,**
- **March 22 – August 22.**

Due to the COVID-19 pandemic, probably a limited number of packages will be used during the first edition.

ERN ACADEMY

The ERN Academy is a tool which wil facilitate the activity of the ERN Knowledge Generation activity

The ERN Academy involves the following components:

- etraining/elearning Moodle-based environment and support to the content generators (the ERNs) to produce the deliverables and manage the system
- Repository-library of all the “products” of the ERNs (the ones included under the agreed taxonomy and any other produced by the ERNs) with a powerful search engine and structure
- Integrated web conferencing system for the training and learning activities
- A central IT and development support and helpdesk for all the activities and needs ERN Academy.

The Project has two stages:

1. Pilot and design phase:

· An initial development (piloting) phase in which the idea is to develop the structure, identify the ERNs needs, type and number of technical and human support, volume and type of activities etc

The ERN Academy project should start small and grow once the model and structure is tested and mature.

· The "pilot" phase include three ERNs: MetabERN, ERKNet and EpiCARE

2. An implementation phase once the model, technical a support needs are identified and developed.

Tools needed

A strong search engine for the library
An index divided by topics
A tool for virtual simulations
A live-streaming tool (would require a strong security system in case of live-streams)

European Reference Networks | ERN GUIDELINES

European Reference Network:
Clinical Practice Guidelines and Clinical Decision Support Tools

TENDER Nº SANTE/2018/B3/030 I April 2021

The ERN Guidelines Programme

Juan-Antonio Blasco MD, MPH
Lourdes González Bermúdez PhD
ERN Guidelines | Coordination Team
Health and Progress Foundation
Seville, Spain

Presentation for BoMS

European Commission

European Reference Networks

ERN Guidelines

Coordinators' Team Meeting

Background

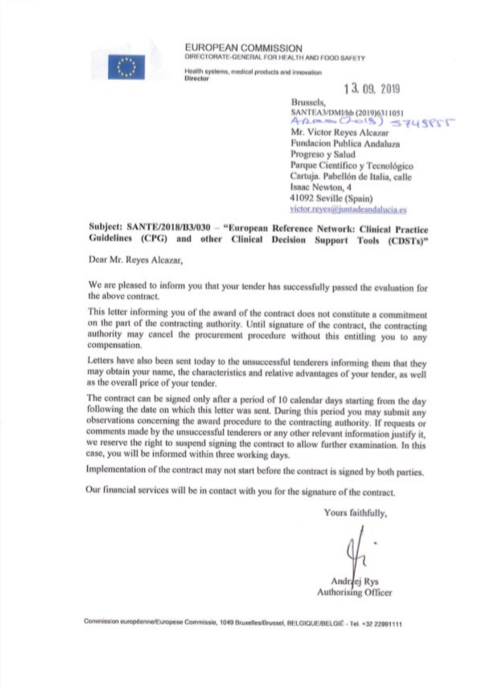
A consortium of 5 Health Technologies Assessment Agencies and 2 Specialized Health Training Institutions won the EC Tender Nº SANTE/2018/B3/030.

In January 2020 the Project officially kicked off.

Objectives:

To provide assistance to the ERNs and their healthcare providers in the process of development, appraisal and implementation for CPGs and CDSTs

To improve the capacity of the Networks in their task to produce and adhere to high quality CPGs and CDSTs



EUROPEAN COMMISSION
DIRECTORATE-GENERAL FOR HEALTH AND FOOD SAFETY

Health systems, medical products and innovation
Director

13. 09. 2019

Brussels,
SANTEA3/DMI/hb (2019)6311051
Ares(2019) 5745855

Mr. Victor Reyes Alcazar
Fundacion Publica Andaluza
Progreso y Salud
Parque Científico y Tecnológico
Cartuja. Pabellón de Italia, calle
Isaac Newton, 4
41092 Seville (Spain)
victor.reyes@juntadeandalucia.es

Subject: SANTE/2018/B3/030 – "European Reference Network: Clinical Practice Guidelines (CPG) and other Clinical Decision Support Tools (CDSTs)"

Dear Mr. Reyes Alcazar,

We are pleased to inform you that your tender has successfully passed the evaluation for the above contract.

This letter informing you of the award of the contract does not constitute a commitment on the part of the contracting authority. Until signature of the contract, the contracting authority may cancel the procurement procedure without this entitling you to any compensation.

Letters have also been sent today to the unsuccessful tenderers informing them that they may obtain your name, the characteristics and relative advantages of your tender, as well as the overall price of your tender.

The contract can be signed only after a period of 10 calendar days starting from the day following the date on which this letter was sent. During this period you may submit any observations concerning the award procedure to the contracting authority. If requests or comments made by the unsuccessful tenderers or any other relevant information justify it, we reserve the right to suspend signing the contract to allow further examination. In this case, you will be informed within three working days.

Implementation of the contract may not start before the contract is signed by both parties.

Our financial services will be in contact with you for the signature of the contract.

Yours faithfully,

Andrzej Rys
Authorising Officer

Commission européenne/Europese Commissie, 1049 Bruxelles/Brussel, BELGIQUE/BELGIË - Tel. +32 22991111

Consortium members

Coordination

Fundación Pública Andaluza Progreso y Salud (**FPS**)

HTAs

Agencia de Evaluación de Tecnologías Sanitarias (**AETSA-FPS I+i**)
Fundación Vasca de Innovación e Investigación Sanitarias (**BIOEF**)
Instituto Aragonés de Ciencias de la Salud (**IACS**)
Agència de Qualitat i Avaluació Sanitàries de Catalunya (**AQuAS**)
Fundación Canaria Instituto de Investigación Sanitaria de Canarias (**FIISC**)

Training

Escuela Andaluza de Salud Pública (**EASP**)
Universitat Oberta de Catalunya (**UOC**)

Fundación Progreso y Salud
CONSEJERÍA DE SALUD Y FAMILIAS

Agencia de Calidad Sanitaria de Andalucía
CONSEJERÍA DE SALUD Y FAMILIAS

AETSA
Evaluación de Tecnologías Sanitarias de Andalucía

bioef
berrikuntza + ikerketa + osasuna eusko fundazioa
fundación vasca de innovación e investigación sanitarias

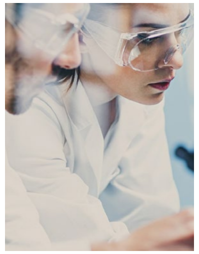
IACS Instituto Aragonés de Ciencias de la Salud

Agència de Qualitat
i Avaluació Sanitàries de Catalunya

FIISC

Escuela Andaluza de Salud Pública
CONSEJERÍA DE SALUD Y FAMILIAS

UOC Universitat Oberta de Catalunya



WORK PACKAGES

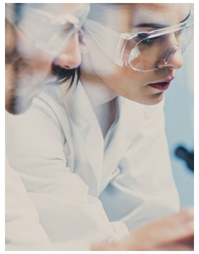
The Project is composed by 4 Work Packages:

WP-A **Governance**

WP-B **Methodology**

WP-C **Training**

WP-D **Development**



WORK PACKAGE A | Governance

CURRENT STATUS OF EXPERT PANELS AND ADVISORY BODY:

During the first semester of 2020 a literature review on existing models and EU Survey were already carried out for the constitution of the AB and EP.

Advisory Body

The **Advisory Body members have already been selected.** The first meeting took place on the 14th January.

Expert Panels

The model to create the Expert Panels is approved. The first meeting with the coordinators of the ERNs to create the EP took place in February.

Microsoft Teams has been chosen as the virtual space system for the Advisory Body and, eventually, for the Expert Panels.

WORK PACKAGE B | Methodology

STATUS:

A literature review and EU Survey on methodology have been carried out and delivered to the EC, identifying methodologies and tools used and recognized at International level for the development, appraisal and implementation of CPGs and CDSTs.

The Methodological Manual and Toolkit for the development, appraisal, adaptation and implementation of CPGs and CDSTs is finalised.

WORK PACKAGE C | Training

STATUS:

A EU-Survey was conducted on training needs regarding CPGs and CDSTs as well as training styles preferences among ERN health professionals.

The training scheme and the plan have already been approved for the **two levels of training: advanced and basic-intermediate.**

- **ToT:** March 2021
- **Training:** April 2021

Virtual platform under preparation.

WORK PACKAGE D | Development

STATUS:

An EU-Survey to the ERNs to define goals and planning for the development and appraisal of CPGs and CDSTs was carried out.

In June 2021 the Expert Panels will begin to develop, appraise and implement the CPGs and CDSTs with the support of the ERN Guidelines Consortium.

In May-June 2021 set up individual meeting between the Coordination Team and the 24 ERNs in order to hear their needs and starting working on CPGs and CDSTs development

Future Steps

1. To improve our ERN Guidelines' communication strategy

↓

Individual meetings between the Coordination Team and the 24 ERNs Coordinators

↓

What are their needs?	Which topics will be selected?	What are their timelines?
To provide specific support in the development the guidelines	To prioritize a subset of main topics for development the guidelines	To align the timing of the Coordinator Team with each ERN for development the guidelines

Future Steps

- Training programme, more flexible in terms of given access to ERN Members.
- Open training programme (stand-alone training) to all interested members for indefinite period of time (EC support platform/Academy for this purpose).
- Patients/representatives shall be enrolled in the training program according to ERNs needs. Patients with no previous experience on CPGs can take the basic training.

European Reference Networks | ERN GUIDELINES

Contact info:

FPS-AETSA
ErnGuideLinesCoordination.fps@juntadeandalucia.es
Fundación Pública Andaluza Progreso y Salud (FPS)
Avd. Américo Vespucio 15
Edificio S-2 CP.41092 Sevilla

☎ +34 955 006 636

European Commission

European Reference Networks

ERN Professional Mobility Programme

- **3 editions planned: April-July 2021, September 2021-January 2022, March-July 2022**
- ERNs have defined priorities and selection criteria for Mobility Programme
- Changes in planning due to Covid-19 uncertainty and restrictive measures across Europe.
- Most ERNs postponing visits to 2nd and 3rd editions.
- 8 ERNs confirmed willingness to participate in 1st edition; 2 ERNs have prepared visits (10 visits)
- Standard visits will last for 5 days; participants will receive 200€ daily allowance per working day.
- Ecorys will provide administrative and logistical support to visitors before, during and after the visit.

ERN and EHDS Academy

- Centralized hosting, support and helpdesk
- Content repository with search engine
- Integrated support for:
    - Discussion fora
    - Web conferencing

ERN and EHDS Academy

- Status:
    - Financed by the EU4Health Program
    - Managed by ~~CHAFEA~~ HADEA
    - First prototype: ~~Q2 2021~~ Q4 2021
    - Pilot content providers: ERKNet, MetabERN, EpiCare

Report from Working group on legal and ethical issues

European
Reference
Networks

European
Commission

UMC Utrecht
Wilhelmina Children's Hospital

Report for WG LES for ERN group meeting

ERN WG-LES

Nico Wulffraat and Helene Kääriänen (co-chairs); Romain Alderweireldt, Cinzia Bellettato, Joan Brunet, Hélène Dollfus, Bruno Fonteyn, Nicoline Hoogerbrugge, Anne-Sophie Lapointe, Inès Palanca, Jean-Philippe Plançon, Muriel Rogasik, Maurizio Scarpa, Corine van Lingen, Anne Vergison and Kléa Vyshka

April 2021

Activities of the WG LES

- **Preparation of documents** (addendum to BOMS statement, Policy document managing COI; ERN disclosure form personal interests; Code of ethical conduct to be developped)
- **Legal issues** between ERNs and HCP (see earlier minutes CG meeting november 2020)
- **Preparation of discussion with Pharma**: survey on registry activity of ERNs

BOMS documents (1)

Addendum to the Statement of the ERN Board of Member States on European Reference Networks (ERNs) & industry (sept 2020).

- Stating the restrictions for relations with industry (must be based on projects only, no direct funding for management allowed?)
- The Need of a Conflict of Interest form for all healthcare providers (including governance)

ERN Conflict of interest forms

- Disclosure form for individual health care providers
  - Text is accepted but governance is not clear. Should be under control of the individual HCP and stored at that site. Alternative: Upload of forms in EU health data space?
  - See also CG minutes(nov 25, 2020): “ERNs needed to handle all the forms coming from their members”

How to collaborate with industry?

ERNS play a role for patients and science

- **ERNs will collaborate with industry: the coordinator (or a designated HCP) will interact with industries and report to BOMs**
- **Interactions between HCP and industry will always exist and follow regulations of the HCP as the legal entity**
- **Prepare a survey for industry needs regarding registries**

Conclusions from the registry survey (2020)

- Registry governance by scientific society or institutional body, Pharma not involved,
- Sustainability (pharma essential), Mixed financing
- availability of data for pharma
- Contracts, IRB, Monitoring in place
- Explore a survey for industry based on existing collaboration of registries with industry

Questions

- How to ensure more legal expertise in the working group?
- Is the legal entity of ERNs an issue that the ERN coordinators would like to pursue further and do they mandate the LES working group to explore potential options?
- How to ensure governance of the COI forms

Coffee break

15:10-15:30

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Report from
ERN IT Advisory Group (ITAG)

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Networks

European
Commission

Main actions of the ITAG in 2020/21

- Two formal meetings in 2020, one in 2021
- Five brainstorm meetings with CPMS users
- Two surveys
- Current tasks:
  - Provide feedback for the new CPMS to be launched in Q1 2023
  - Provide feedback for the Virtual Academy to be launched Q4 2021

State of play of current IT tools and activities

- Service Directory (SD)
  - Stable, all affiliated partners uploaded
  - Under testing: external edit role
- Indicators (IND)
  - Stable form; some problems in the data warehouse and reporting tool
  - Data collection of March closed successfully (C19 and Brexit)
  - Next data collection in October – introductory webinar on 15.09.2021

State of play of current IT tools and activities

- HCPA – Healthcare Provider Application
  - Up and running (IAB stage)
  - Ongoing: preparation for data cleaning and migration to the SD
- CPMS – Clinical Patient Management System
  - 1800 panels, new contract to start on 1.06.2021
  - Simplified workflow in production
  - Under analysis: expert groups and streamlined on-boarding
  - On hold: implementation of new customised datasets

The future of CPMS

- IP rights: released under a Creative Commons License (CC BY 4.0)
- Short term (1.06.2021 - 2022/23)
  - Same contractor (OpenApp)
  - Fees:
    - Licensing: global pay per user (no longer packs of 50 users per ERN)
    - Software developments: on a case by case basis (time and means)
    - General management: yearly fee

The future of CPMS

- Long term (beyond 2022/23)
- The same approach:
  - Monolithic
  - Common and custom elements
  - EC/Contractor manages everything
- A new approach:
  - Loosely-coupled architecture
  - Modular and open source
  - Flexible management

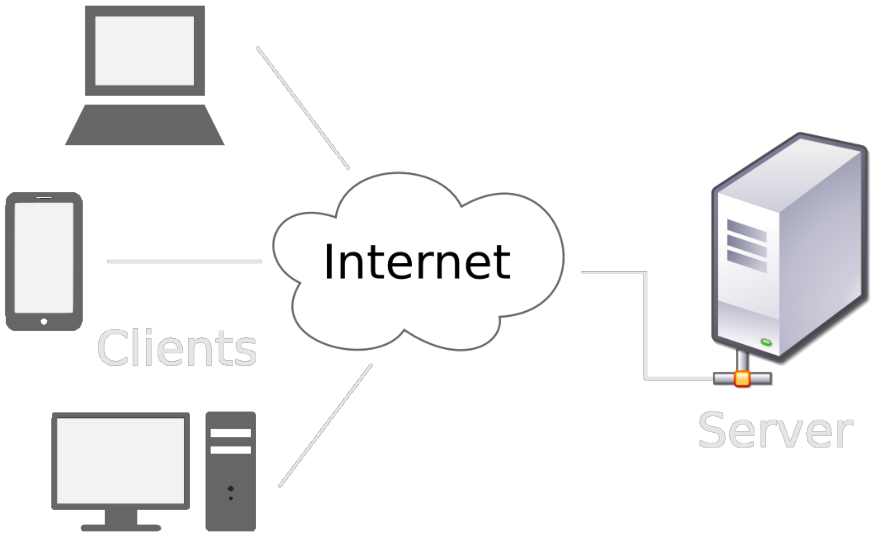
Secure, GDPR compliant, backwards compatible!

The future of CPMS

- Centralised Database and governance
- Modular:
  - Clinical functions (ERNs)
  - Management and Monitoring (ERNs, MS, HCPs, EC)
  - Seamless connection to ERN registries and electronic health records (ERNs, HCPs)

The future of CPMS

- Loosely-coupled architecture:
    - CPMS Server and underlying database management systems
    - Reference clients
    - Customized clients



The future of CPMS

- Open Source:
  - Database structure
  - Application Program Interfaces
  - Code base (reference clients)
- Closed: personal and health data

open source®
initiative

The future of CPMS

- IT Governance:
  - Centralised development and management
    - CPMS Server and underlying databases
    - Application Program Interfaces
    - Reference clients
  - Decentralised development and management
    - Customized clients

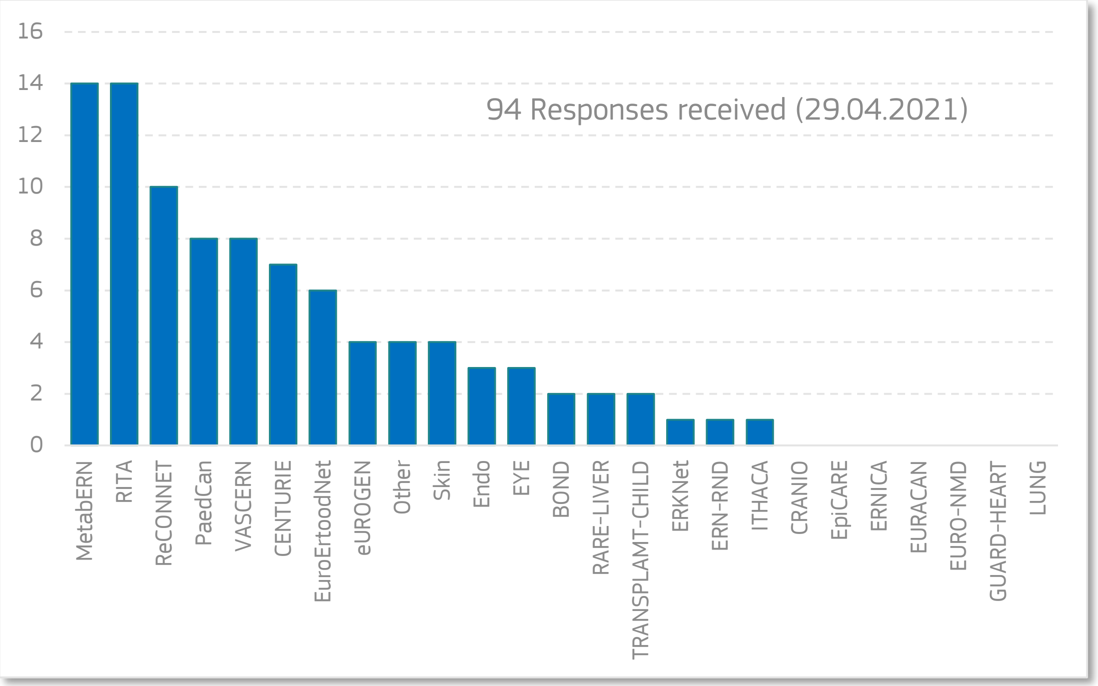
The future of CPMS

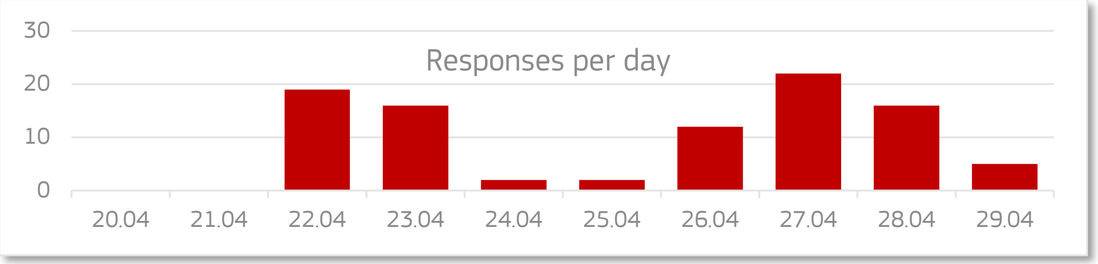
- Security and data privacy:
  - Access based on two-factor authentication
  - Encrypted communications and data storage
  - Consent-based, GDPR compliant
  - Pursuing to the conducted DPIA recommendations

Presentation of DPIA results on 4.05.2021

The future of CPMS

- Preparatory work:
  - 5 brainstorm workshops
  - Survey to the ERN community of users (deadline: 11.05.2021)
- Next step:
  - Incorporate all the input into the technical annex of the tender





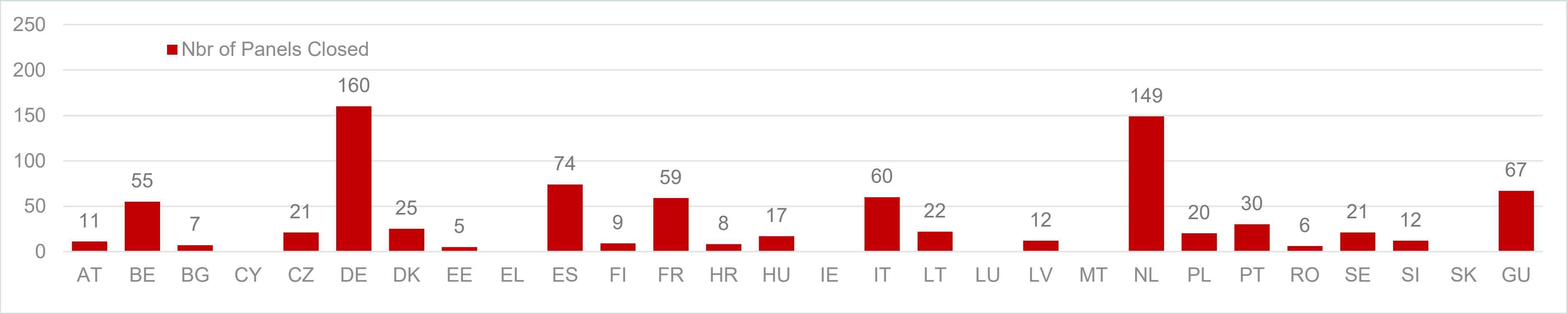
CPMS activity report

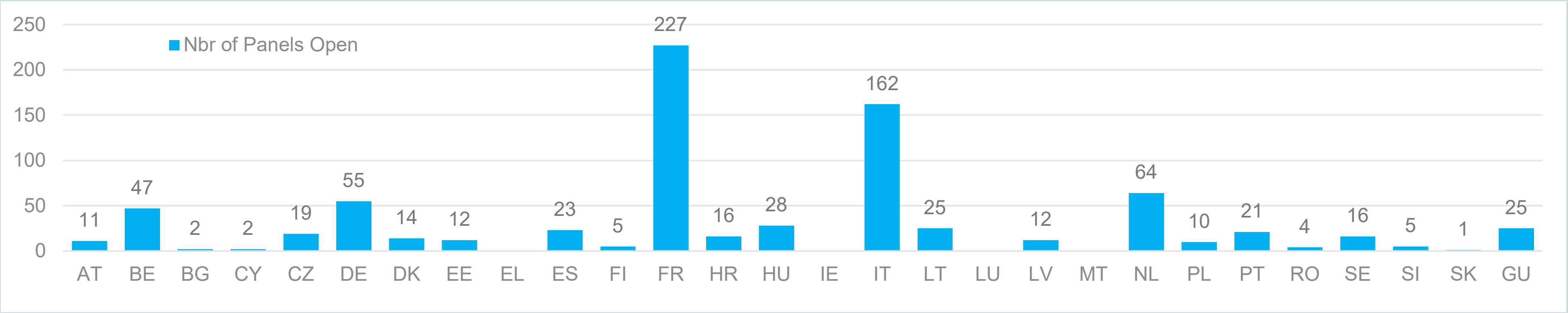
Information point

European Reference Networks

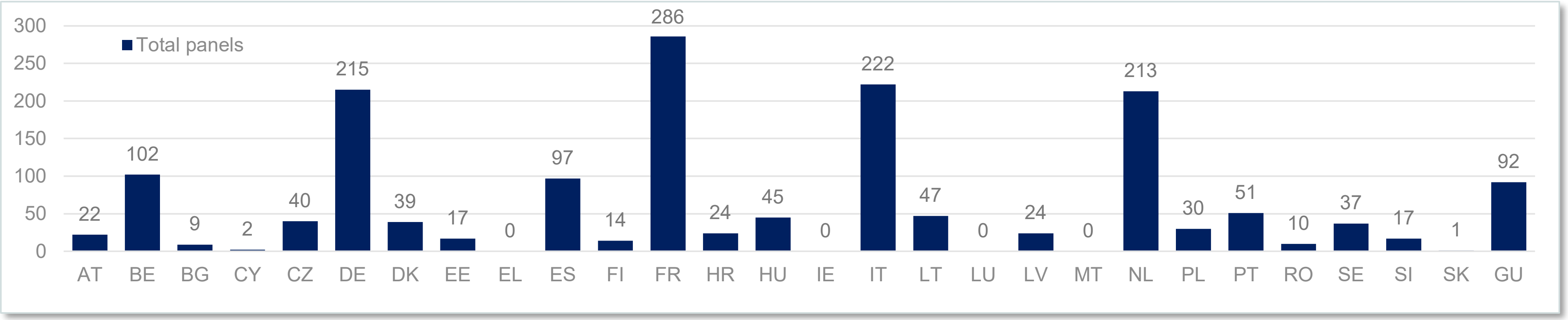
European Commission

CPMS – Closed and open Panels per Country





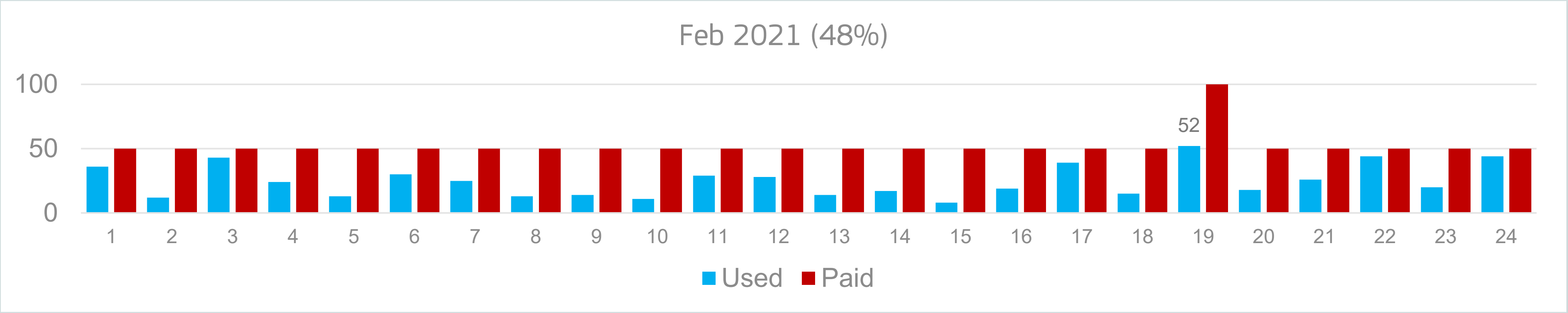
CPMS – Total Panels per Country

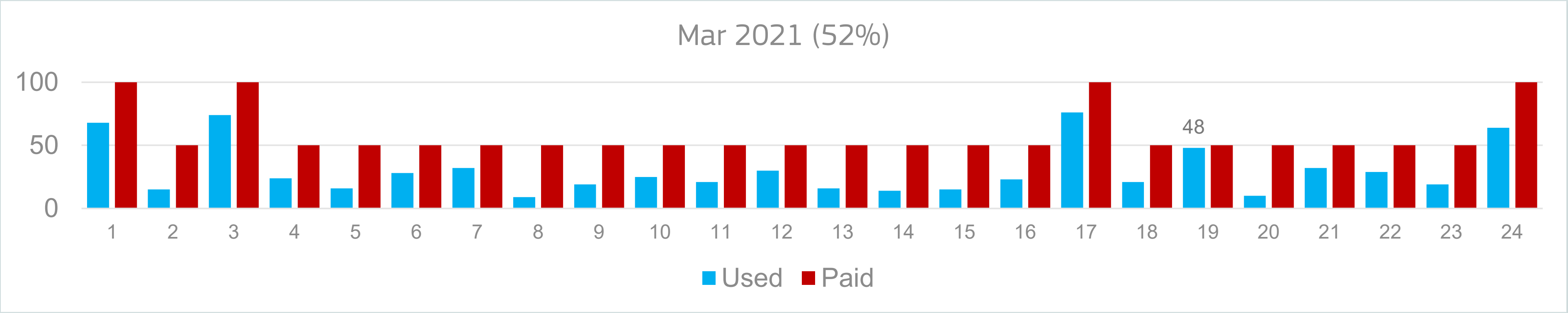


Total panels

1656 + 143 = 1799

CPMS – Licenses per ERN





Beginning of new contract: 1.06.2021

Report from Task Force on ERN registries

European
Reference
Networks

European
Commission

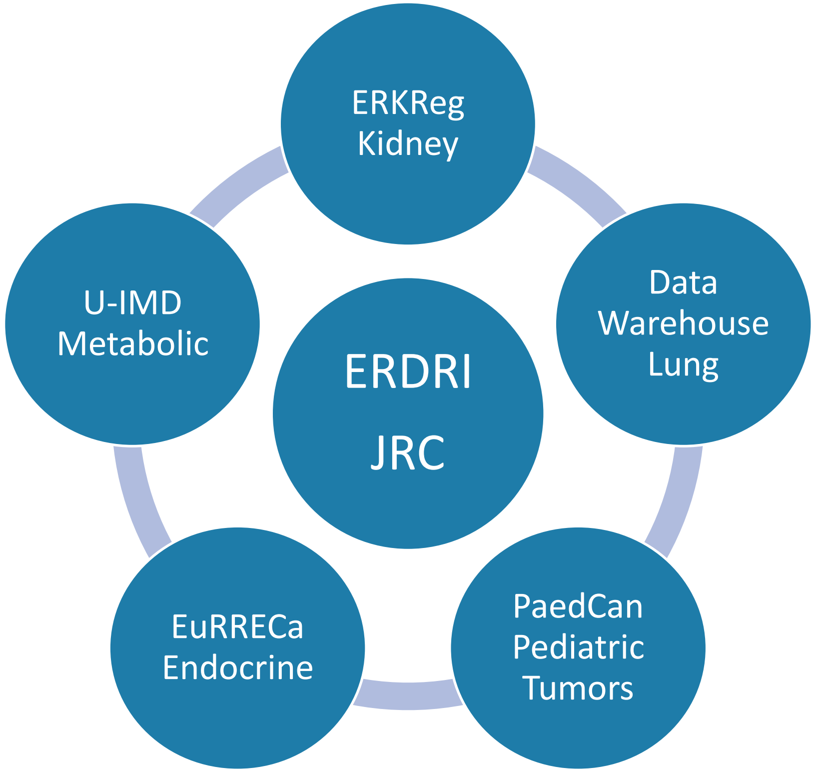
ERN Research: Registry Activities, EJP-RD, ERICA

Franz Schaefer

Center For Rare Kidney Diseases
Heidelberg University Hospital
Germany

ERN-Wide Patient Registries

- EU Health Programme 2018:
  Registries for European Reference Networks
- 5 ERNs funded to develop core registries
- Funding period 2018-2020


ERKReg
Kidney

Data
Warehouse
Lung

PaedCan
Pediatric
Tumors

EuRRECa
Endocrine

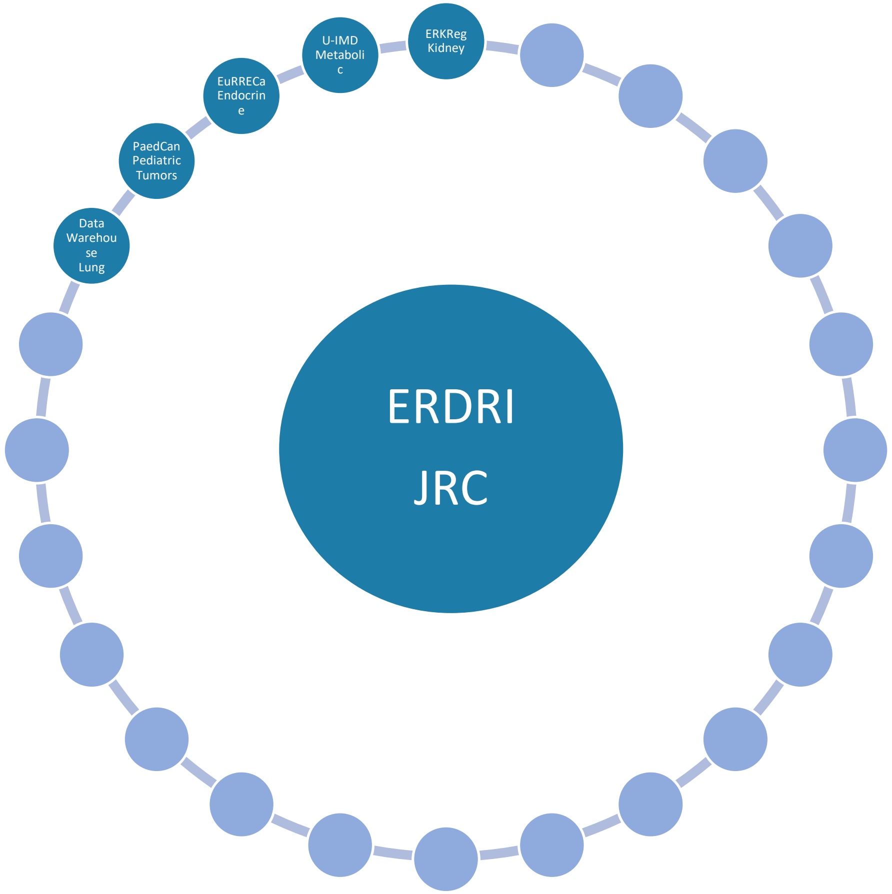
U-IMD
Metabolic

ERDRI

JRC

ERN-Wide Patient Registries

- EU Health Programme 2018:
  Registries for European Reference Networks
- 5 ERNs funded to develop core registries
- Funding period 2018-2020
- Health Programme 2019:
  Funding for ERN wide registries in 19 remaining ERNs
- Funding period 2020-2022



Current Status of ERN Registries (Feb-21)

- **Data dictionaries:**
  6 complete, 14 in progress, 4 about to start

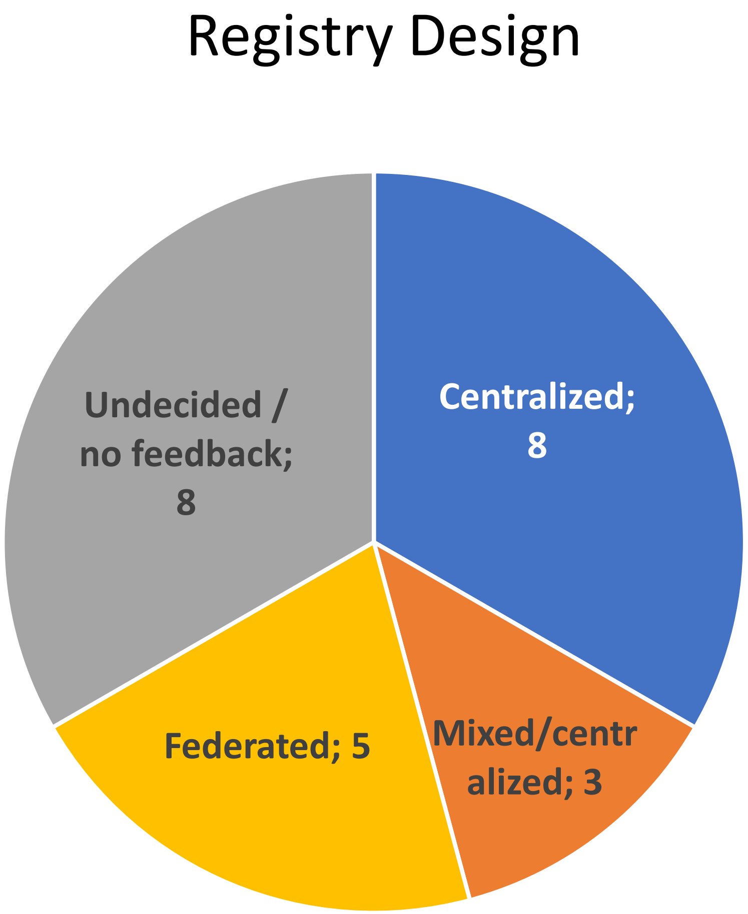
- **Registry programming:**
  7 finished, 8 prototype, 8 in programming, 1 not yet started

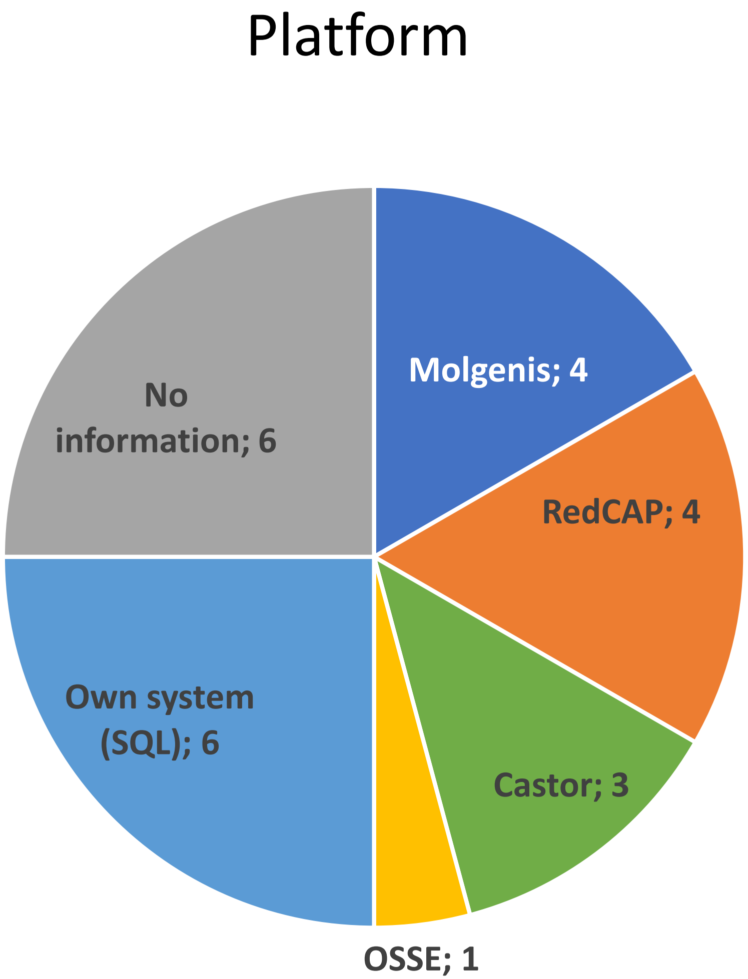
- **Ethics Committee review:**
  5 all approved, 3 most sites submitted, 5 in preparation, 11 waiting for common IC form

- **Patients enrolled:**

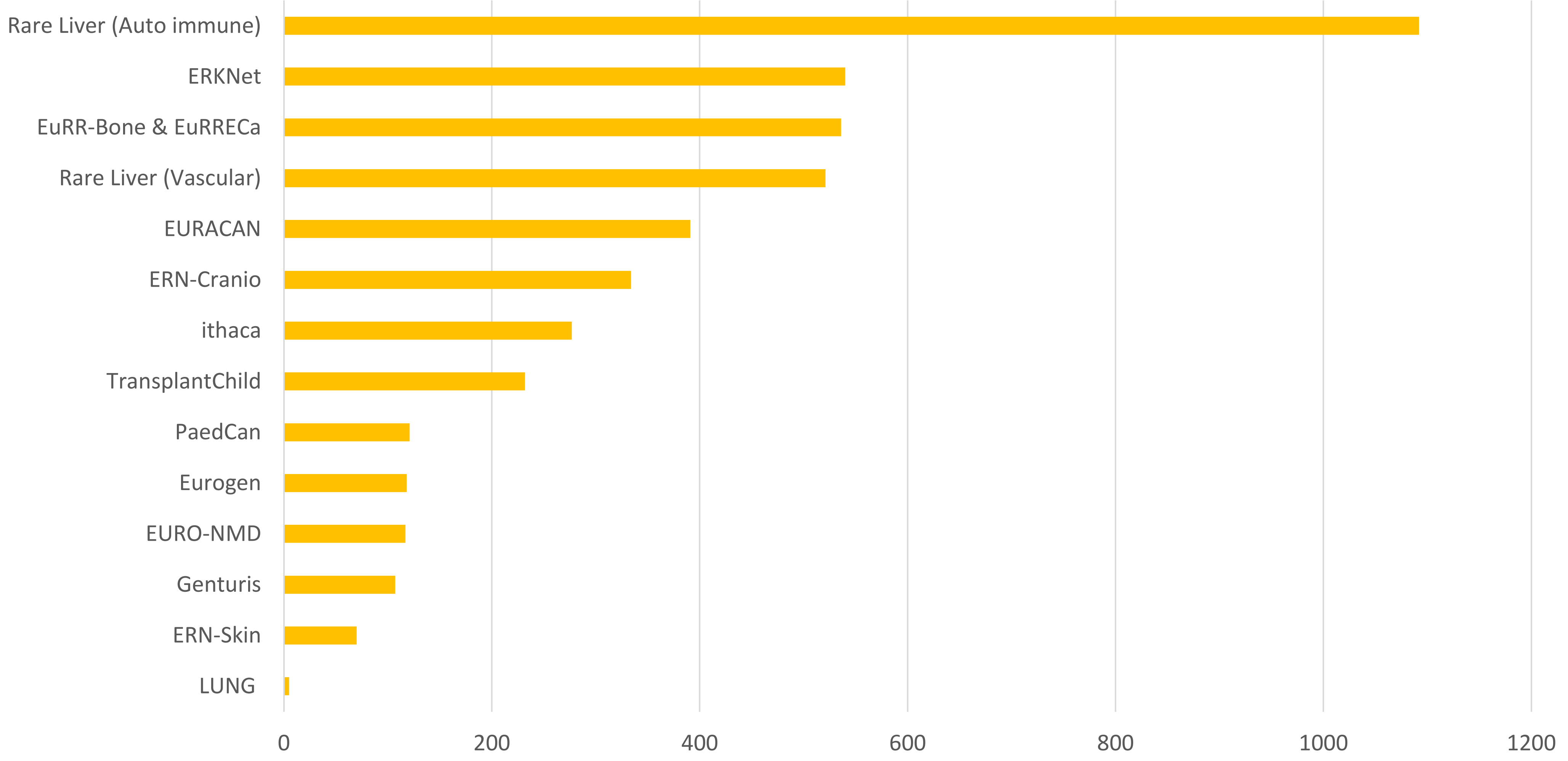
17	<100
4	100-1,000
3	>5,000

Current Status of ERN Registries (Feb-21)



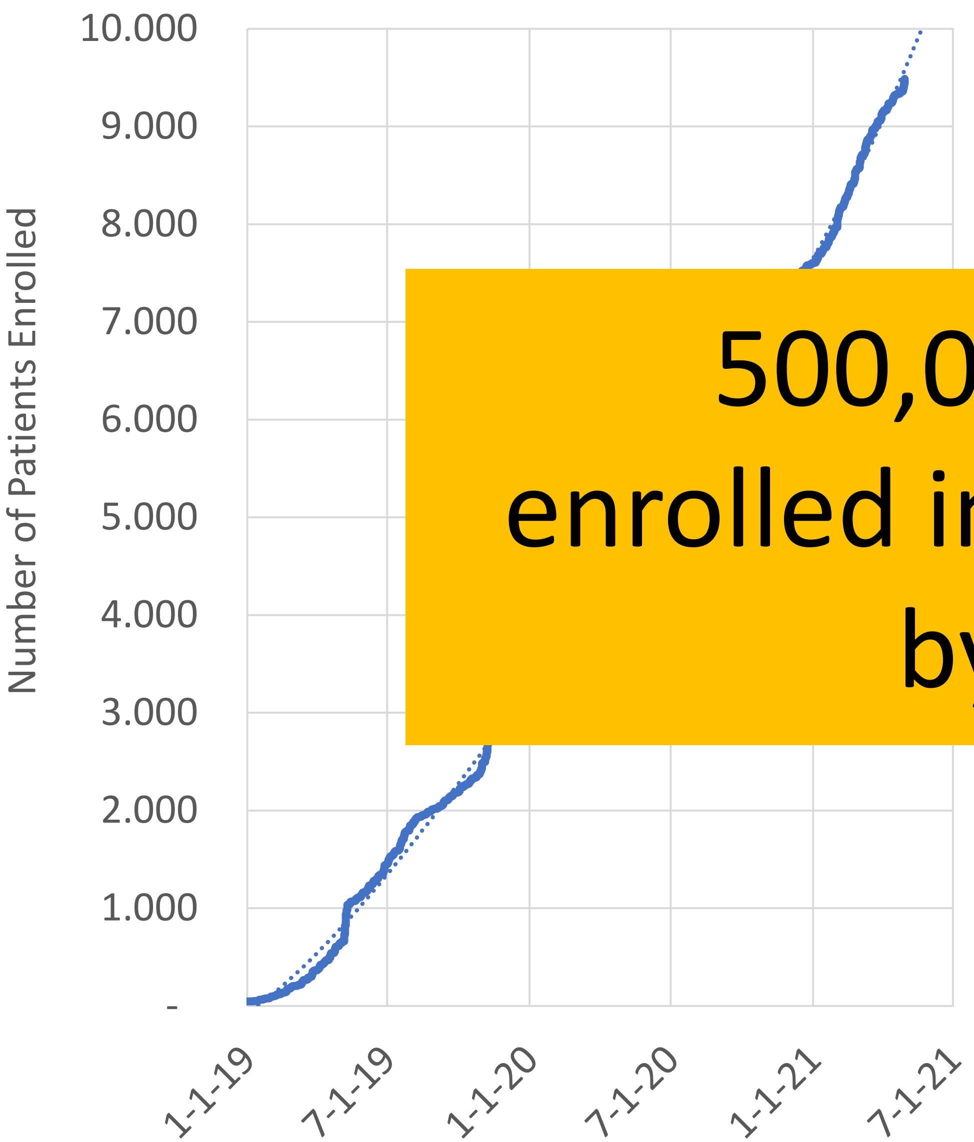


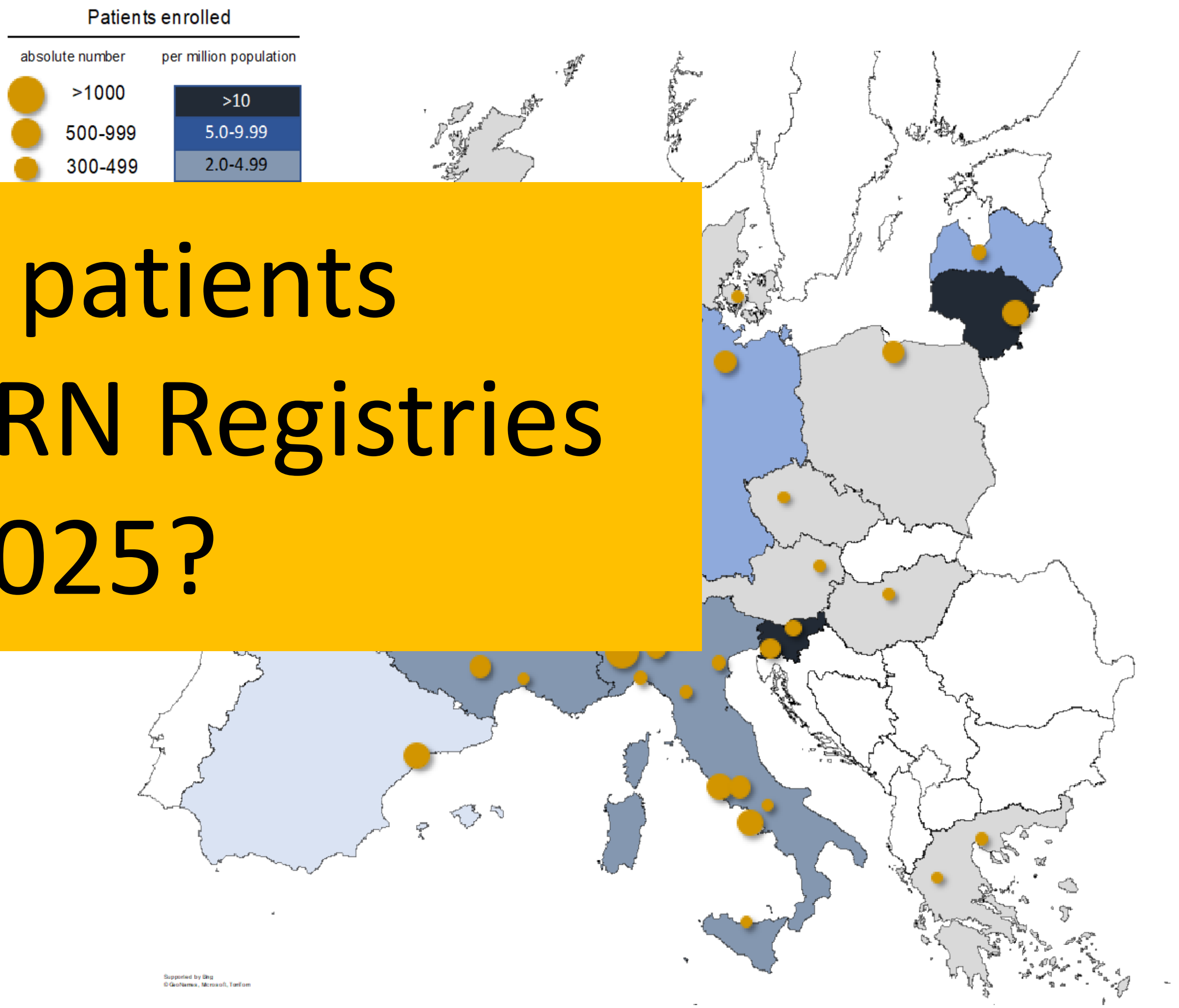
Data Fields per ERN Registry



ERKReg: The European Rare Kidney Disease Registry

ERKReg
The Rare Renal Registry





500,000 patients
enrolled in ERN Registries
by 2025?

EUROPEAN JOINT PROGRAMME RARE DISEASES

P1

WP6
Joint Transnational Calls

WP7
Networking scheme

WP8
RDR Challenges

WP9
Monitoring of funded projects

P2

WP 10
User-driven strategic planning for P2

WP 11
Virtual Platfform for data & resources

WP 12
Enabling sustainable FAIRness

WP 13
Holistic approaches for rare disease diagnostics and therapeutics

P3

WP 14
Training on data management & quality

WP 15
Capacity building and training of patients and researchers

WP 16
Online Academic education course

WP 17
ERN RD training and support programme

WP 18
Development and adaptation of training activities

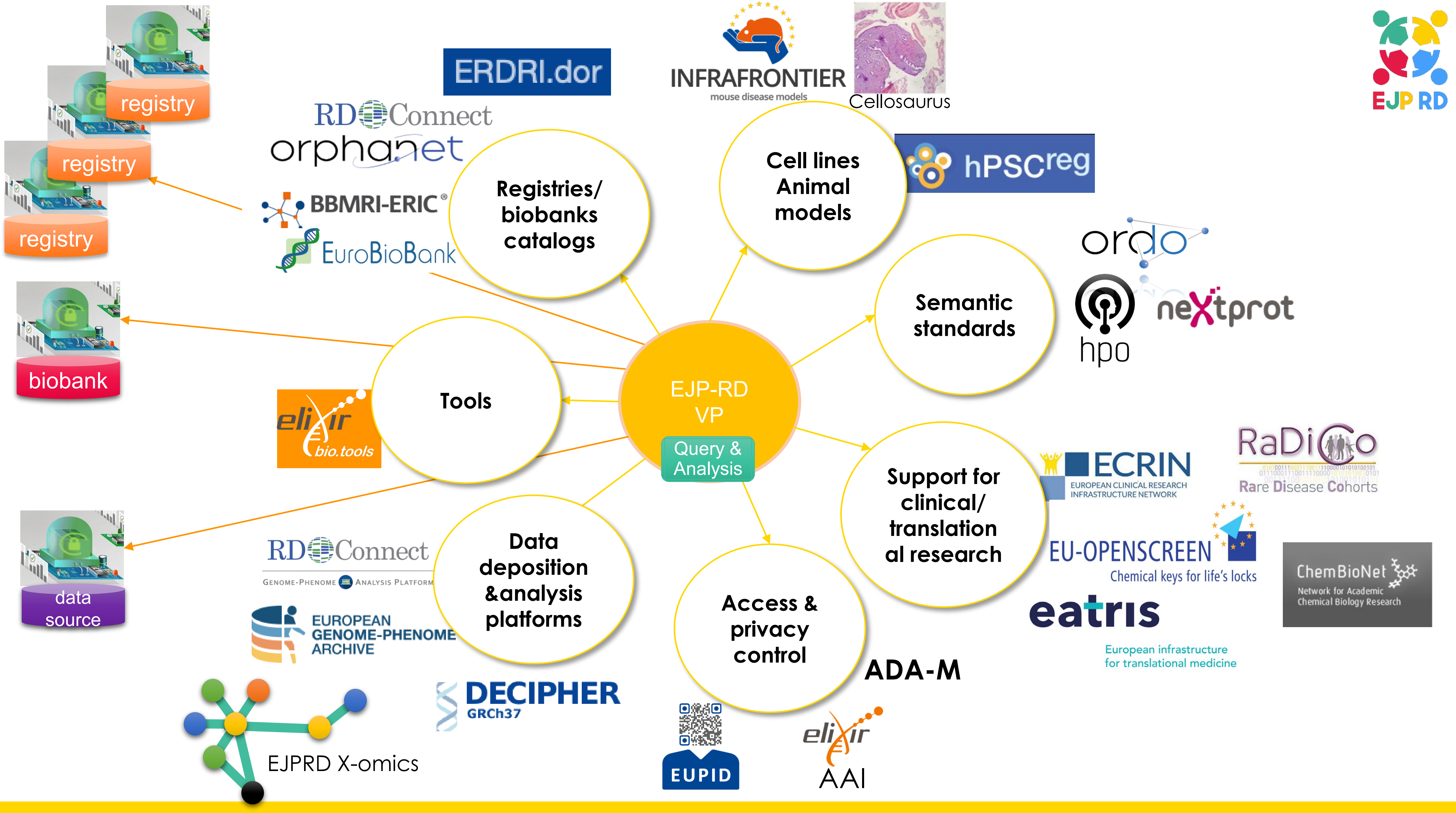
P4

WP 19
Facilitating partnerships and accelerating translation

WP 20
Validation , use and development of innovative methodologies for clinical trials

EJP RD

Funded by the
European Union
GA nº825575



EJP RD

EJP-RD VP

Query & Analysis

Registries/ biobanks catalogs

Cell lines Animal models

Semantic standards

Support for clinical/ translational research

Access & privacy control

Data deposition &analysis platforms

Tools

registry

registry

registry

biobank

data source

ERDRI.dor

RD-Connect

orphanet

BBMRI-ERIC

EuroBioBank

INFRAFRONTIER
mouse disease models

Cellosaurus

hPSCreg

ordo

hpo

neXtprot

ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK

RaDiCo
Rare Disease Cohorts

EU-OPENSCREEN
Chemical keys for life's locks

ChemBioNet
Network for Academic Chemical Biology Research

eatris
European infrastructure for translational medicine

ADA-M

EUPID

elixir AAI

DECIPHER
GRCh37

RD-Connect
Genome-Phenome Analysis Platform

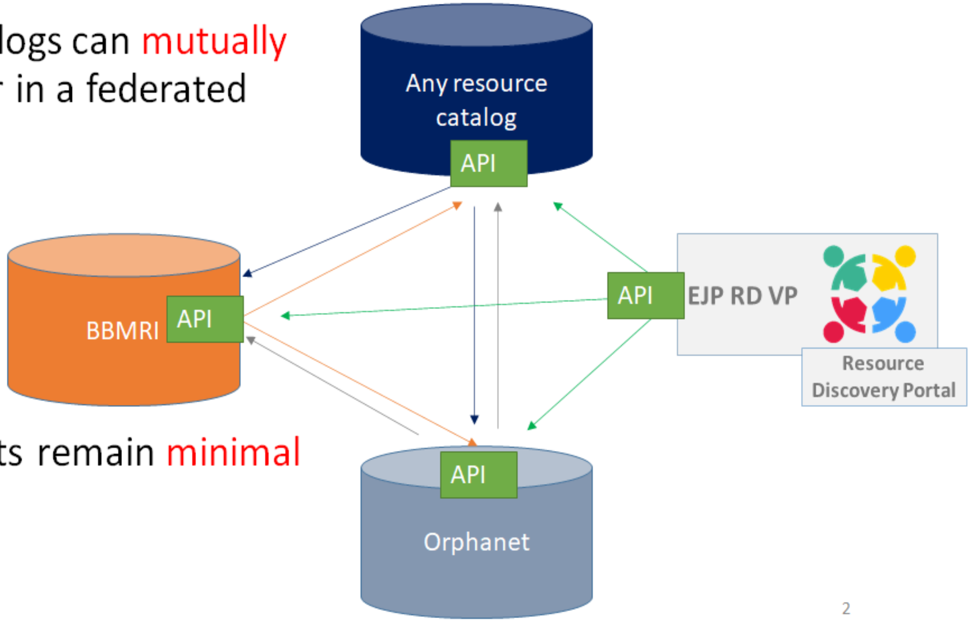
EUROPEAN GENOME-PHENOME ARCHIVE

EJPRD X-omics

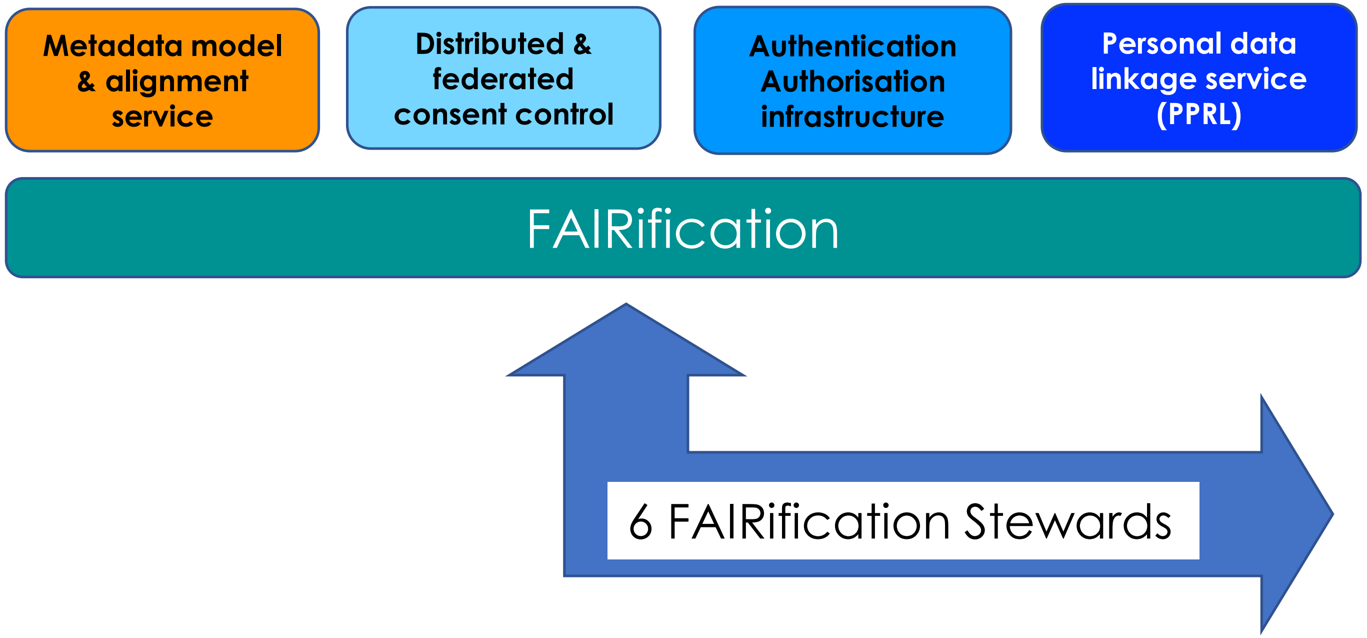
elixir bio.tools

The “Virtual Platform”:
Connecting ERN Registries and Other Resources

- Participating catalogs can mutually search each other in a federated manner
- Query can be initiated at any site
- Transmitted results remain minimal



Pillar 2



Metadata model & alignment service

Distributed & federated consent control

Authentication Authorisation infrastructure

Personal data linkage service (PPRL)

FAIRification

6 FAIRification Stewards

European Rare Disease Registry Infrastructure (ERDRI)

European Directory of Registries (ERDRI.dor)

Overview of rare disease registries in Europe including their characteristics

Central Metadata Repository (ERDRI.mdr)

Database containing the data elements used by rare disease registries

Pseudonymisation tool

Service offering registries at local level the solution for patient pseudonymisation

Pillar 2

Metadata model & alignment service

Distributed & federated consent control

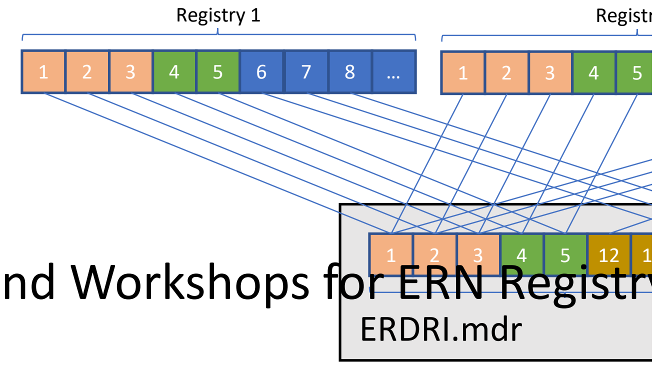
Authentication Authorisation infrastructure

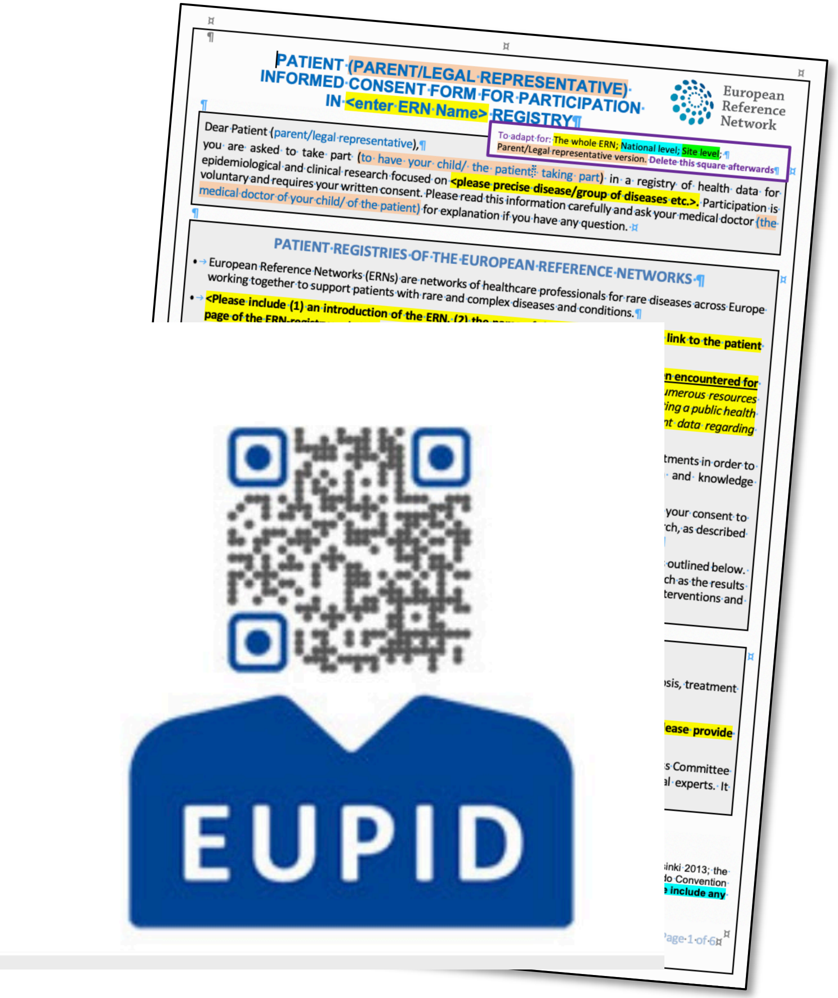
Personal data linkage service (PPRL)

FAIRification

Current Activities:

- Generic Informed Consent form for all registries
- Definition of Cross-ERN Domain Specific Common Data Elements -> DCDE working groups being formed
- Facilitating registries‘ access to EUPID
- Series of Webinars (‚Coffee Rounds‘) and Workshops for ERN Registr




PATIENT (PARENT/LEGAL REPRESENTATIVE) INFORMED CONSENT FORM FOR PARTICIPATION IN <enter ERN Name> REGISTRY

European Reference Network

To adapt for: The whole ERN; National level; Site level;
Parent/Legal representative version. Delete this square afterwards

Dear Patient (parent/legal representative),

you are asked to take part (to have your child/ the patient taking part) in a registry of health data for epidemiological and clinical research focused on **<please precise disease/group of diseases etc.>**. Participation is voluntary and requires your written consent. Please read this information carefully and ask your medical doctor (the medical doctor of your child/ of the patient) for explanation if you have any question.

PATIENT REGISTRIES OF THE EUROPEAN REFERENCE NETWORKS

- European Reference Networks (ERNs) are networks of healthcare professionals for rare diseases across Europe working together to support patients with rare and complex diseases and conditions.
- **<Please include (1) an introduction of the ERN, (2) the**

page of the ERN-registr

link to the patient

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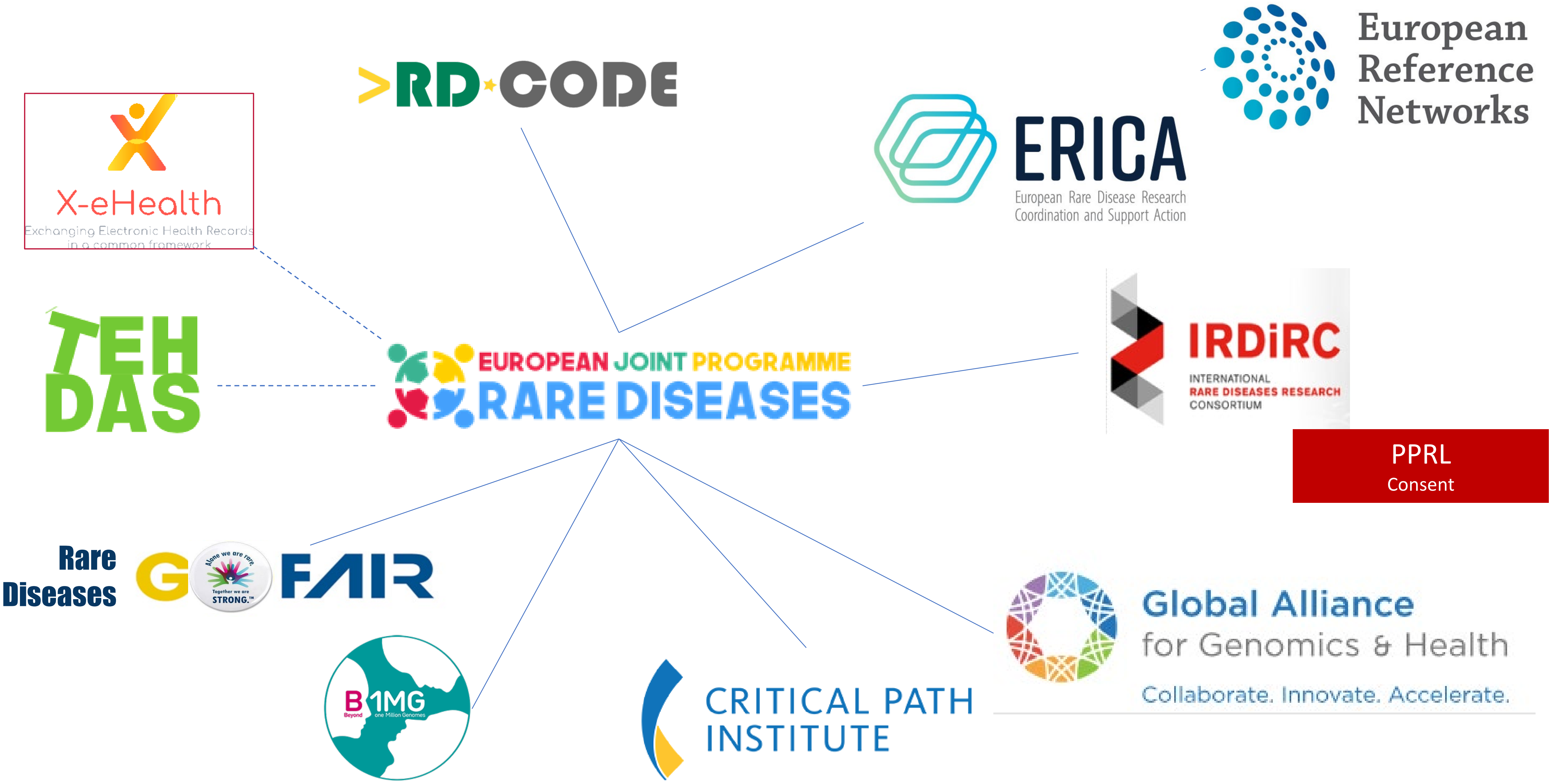
Linking European and Global RD Data Projects



PPRL

Consent

ERN Research Fellowships

Results of 3rd call 2021

New rules: - up to 6 months fellowship duration
- only sending OR receiving institution must be ERN

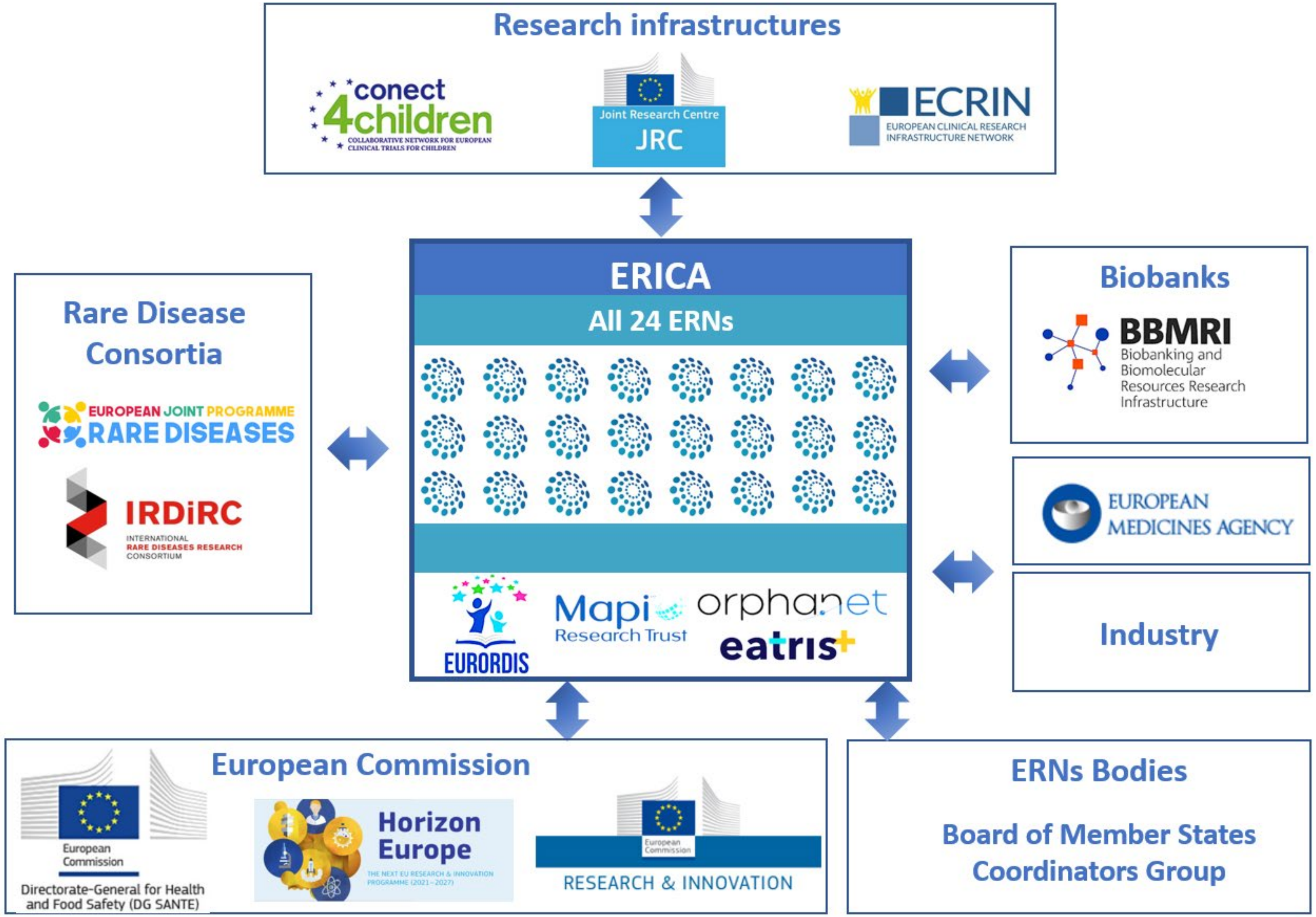
- **25 applications submitted**
- 13/25 asking for 6 months stay
- 12 PhD students, 4 post-docs, 9 MDs in specialist training
- 12 different ERNs involved
- 5/25 non-ERN sending institutions
- 5/25 non-ERN receiving institutions
- Most applicants from Italy (5), NL (4), Turkey (4)
- Most popular host countries: NL (7), Germany (5), UK (5)

ERICA

European Rare Disease Research
Coordination and Support Action

Start of Funding: 01 March, 2021

Positioning of ERICA within the RD research ecosystem



Research infrastructures

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

Joint Research Centre
JRC

ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK

Rare Disease Consortia

EUROPEAN JOINT PROGRAMME RARE DISEASES

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

ERICA

All 24 ERNs

EURORDIS

Mapi Research Trust

orphanet

eatris+

Biobanks

BBMRI
Biobanking and Biomolecular Resources Research Infrastructure

EUROPEAN MEDICINES AGENCY

Industry

European Commission

European Commission
Directorate-General for Health and Food Safety (DG SANTE)

Horizon Europe
THE NEXT EU RESEARCH & INNOVATION PROGRAMME (2021 – 2027)

European Commission
RESEARCH & INNOVATION

ERNs Bodies

Board of Member States
Coordinators Group

ERICA
European Rare Disease Research Coordination and Support Action

WP 2: Data Collection, Integration and Sharing

Leads: Franz Schaefer, Eduardo Granados

Coordination and Support of ERN registry activities

-> WGs for Implementation, ethico-legal issues

- Registry FAIRness
- Efficient data collection and management strategies for ERN registries
- inter-ERN registry research collaborations

Facilitation of biobanking *(-> ERN biobanking WG)*

Contribution to development of EJP RD virtual platform

ERICA
European Rare Disease Research
Coordination and Support Action

WP 3: Patient Centered Research

Leads: Mar Manu Pereira, Ana Rath

- Strategic steering of identification, development and implementation of **patient-centred outcome measures** in ERN-lead clinical research
- Stateof the art of available PCOMs/PROMs and overlap/gap analysis
- Supporting ERNs on implementation of validated instruments for PCOMs/PROMs

ERICA
European Rare Disease Research
Coordination and Support Action

WP 4: Clinical Trial Support

Leads: Luca Sangiorgi, Ralf-Dieter Hilgers

- WP4 Expert Working Group
- ERICA specific webpage on RD clinical trials
- RD Trial Workshops
- Interaction with stakeholders essential in facilitating ERN clinical trials
- Framework for patient engagement

ERICA
European Rare Disease Research
Coordination and Support Action

WP 5: Translation and Innovation

Leads: Ruth Ladenstein, Anton Ussi

- Mapping and cataloguing
- Educational & best practice exchange workshops
- Educational Webinars
- Innovation expert working group

ERICA
European Rare Disease Research
Coordination and Support Action

WP 6 : Integration, Outreach & Dissemination

Leads: Alberto Pereira, Maurizio Scarpa

- Coordination of dissemination and communication
- Interactive web-based platform for ERN research
- Networking

ERICA
European Rare Disease Research
Coordination and Support Action

ERICA

European Rare Disease Research
Coordination and Support Action

Kickoff Meeting:
May 22-23

ERICA 1st General Assembly

27th May	DAY 1
	Plenary session
10:00 – 10:15	Welcome and the general presentation of the project
10:15 – 10:30	Presentation of the Advisory Boards
10:30 – 10:50	**Hélène Le Borgne, EC- DG Research** "Vision on challenges and opportunities of the ERICA projects from the EC- DG Research perspective"
10:50 – 11:10	**Yann Le Cam, EURORDIS** "Vision of rare disease research in the next 15 years from the Rare 2030 perspective
11:10 – 11:30	**Daria Julkowska, EJP RD** "Vision on the challenges and opportunities of the ERICA project in relation to EJP RD"
11:30 – 11:45	Break
11:45 – 12:45	WP1-6 Introductions (10 min each)
12:45 – 13:00	Q&A
13:00 – 14:00	Lunch break
	WP Specific sessions
14:00 – 15:00	EWG2 **'Data Collection, Integration and Sharing'**
15:00 – 15:15	break
15:15 – 16:15	EWG3 **'Patient Centered Research'**
16:15 – 16:30	break
16:30 – 16:45	Plenary wrap up day 1
28th May	DAY 2
	Plenary session
10:00 – 10:15	Opening day 2 and recap day 1
10:15 – 10:35	**Simona Martin, JRC** "Synergies between the EU RD Platform and ERICA"
10:35 – 10:55	**Lucia Monaco, IRDiRC** "Vision on rare disease research from the IRDiRC perspective"
10:55 – 11:15	**Matthias Uhlén, KTH Swedish Royal Institute of Technology** "the examples of new ways of research and innovation programmes that open new ways to therapy: opportunities for ERICA (tbc)
11:15 – 11:30	break
	WP Specific sessions
11:30 – 12:30	EWG4 **'Clinical Trial Support'**
12:30 – 12:45	break
12:45 – 13:45	EWG5 **'Translation and Innovation'**
13:45 – 14:30	Lunch break
14:30 – 15:30	EWG 6 **'Integration, Outreach & Dissemination'** (plenary)
15:30 – 15:45	Plenary: Concluding remarks of EWGs and Take-home messages

European Health Data Space and ERN registries

Discussion point

European Health Data Space
and ERN registries

30 April 2021 @ ERNs Coordinators Group

DG SANTE

European Reference Networks and Digital Health

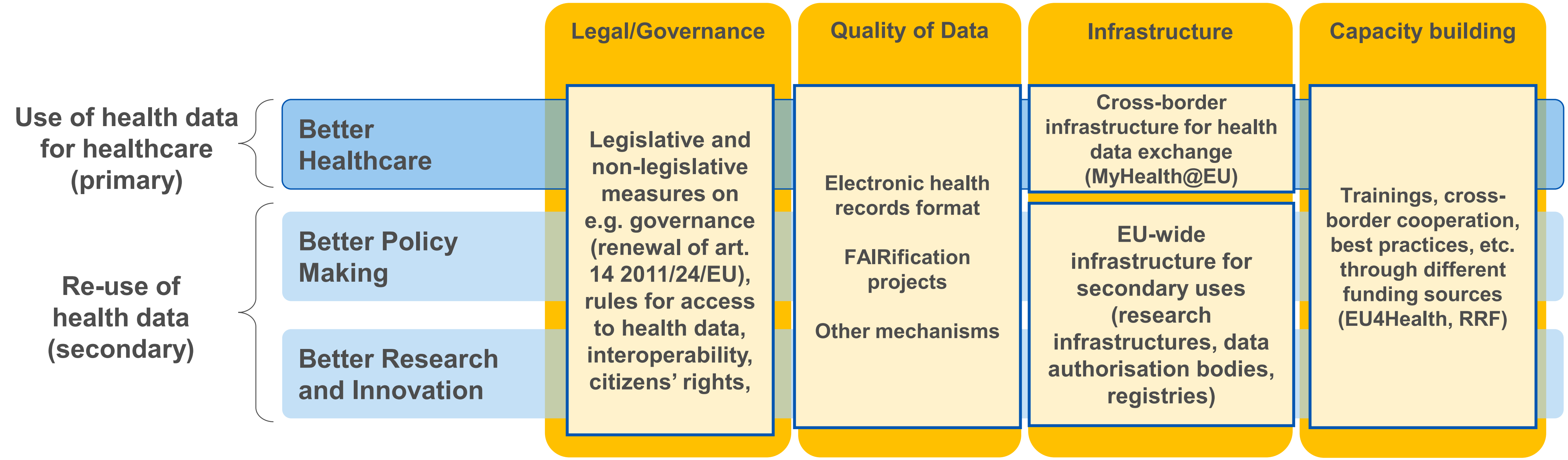
Mission letter of
Stella Kyriakides
Commissioner for Health

“We need to make the most of the potential of e-health to provide high-quality healthcare and reduce inequalities. I want you to work on the creation of a ***European Health Data Space*** *to* ***promote health-data exchange*** *and* ***support research*** *on* ***new preventive strategies****, as well as on* ***treatments, medicines, medical devices and outcomes****. As part of this, you should ensure* ***citizens have control*** *over their own* ***personal data****.”*

How to accomplish such mission?

Primary and secondary uses of health data

Timely and harmonised rules for the *exchange of* and *access to* health data for different use cases, e.g. research on cancer, pharmacovigilance, etc., but also for healthcare provision.



Use of health data for healthcare (primary)

Re-use of health data (secondary)

Better Healthcare

Better Policy Making

Better Research and Innovation

Legal/Governance

Legislative and non-legislative measures on e.g. governance (renewal of art. 14 2011/24/EU), rules for access to health data, interoperability, citizens' rights,

Quality of Data

Electronic health records format

FAIRification projects

Other mechanisms

Infrastructure

Cross-border infrastructure for health data exchange (MyHealth@EU)

EU-wide infrastructure for secondary uses (research infrastructures, data authorisation bodies, registries)

Capacity building

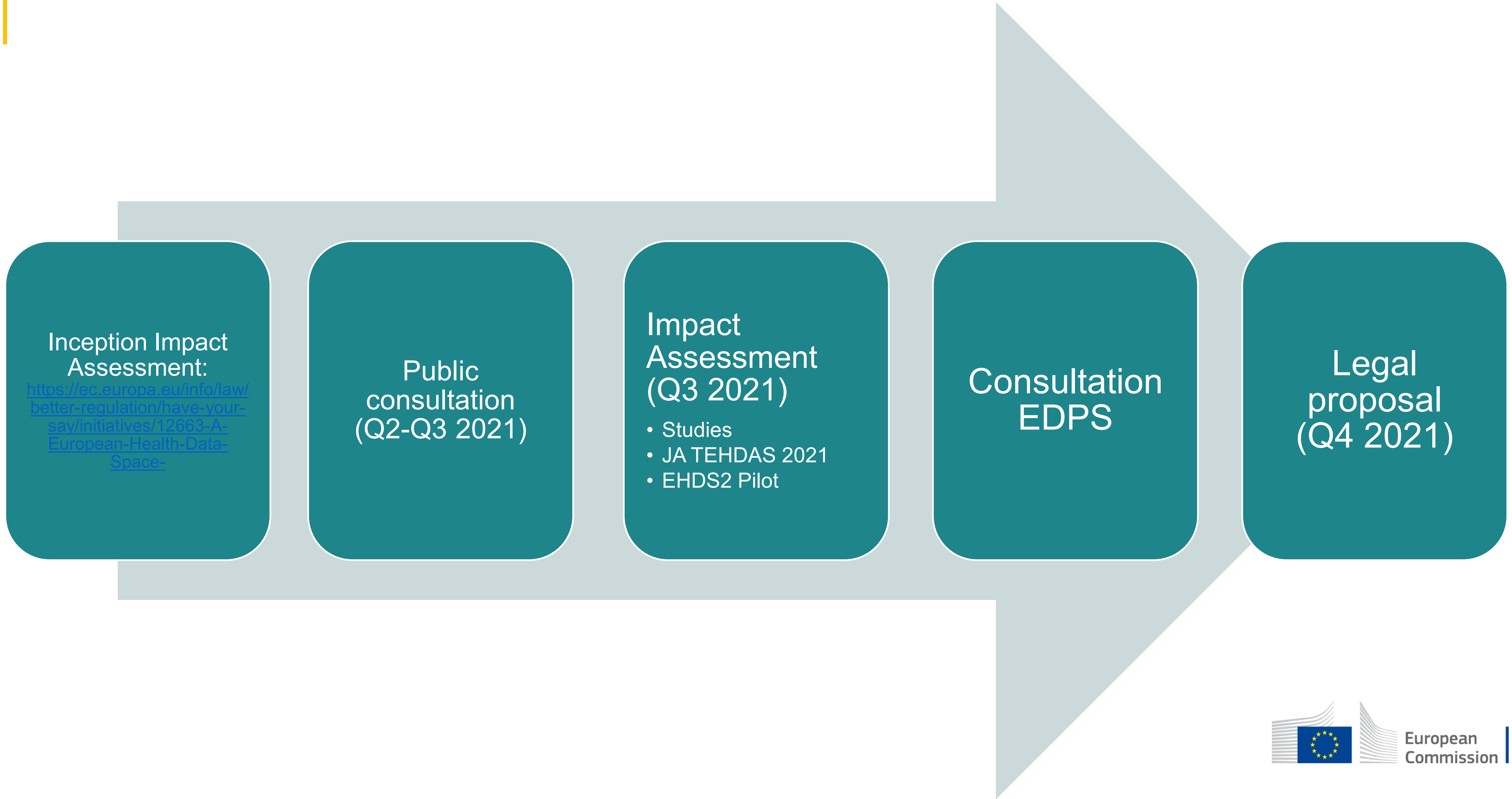
Trainings, cross-border cooperation, best practices, etc. through different funding sources (EU4Health, RRF)

European Commission

Studies supporting EHDS initiative

Study on assessment of MS rules on health processing in the light of GDPR	• Published on 12 February 2021: https://ec.europa.eu/health/sites/health/files/ehealth/docs/ms_rules_health-data_en.pdf
Regulatory gaps study (obstacles / drivers and costs / benefits)	• Workshops and stakeholders consultation carried out • Report to be finalised in June 2021
Infrastructure study (designing options for EHDS1 and EHDS2)	• Started April 2021 – 5 months duration
Impact Assessment study	• Assessing offers received • To start in May 2021 – 5 months duration

EHDS: regulation roadmap



Inception Impact Assessment: https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12663-A-European-Health-Data-Space-

Public consultation (Q2-Q3 2021)

Impact Assessment (Q3 2021)

- Studies
- JA TEHDAS 2021
- EHDS2 Pilot

Consultation EDPS

Legal proposal (Q4 2021)

EHDS2: pilot: motivation

Targets

2021: Pilot infrastructure

2025: EU-wide infrastructure

Testbed (proof of concept)

Governance

IT and data infrastructure

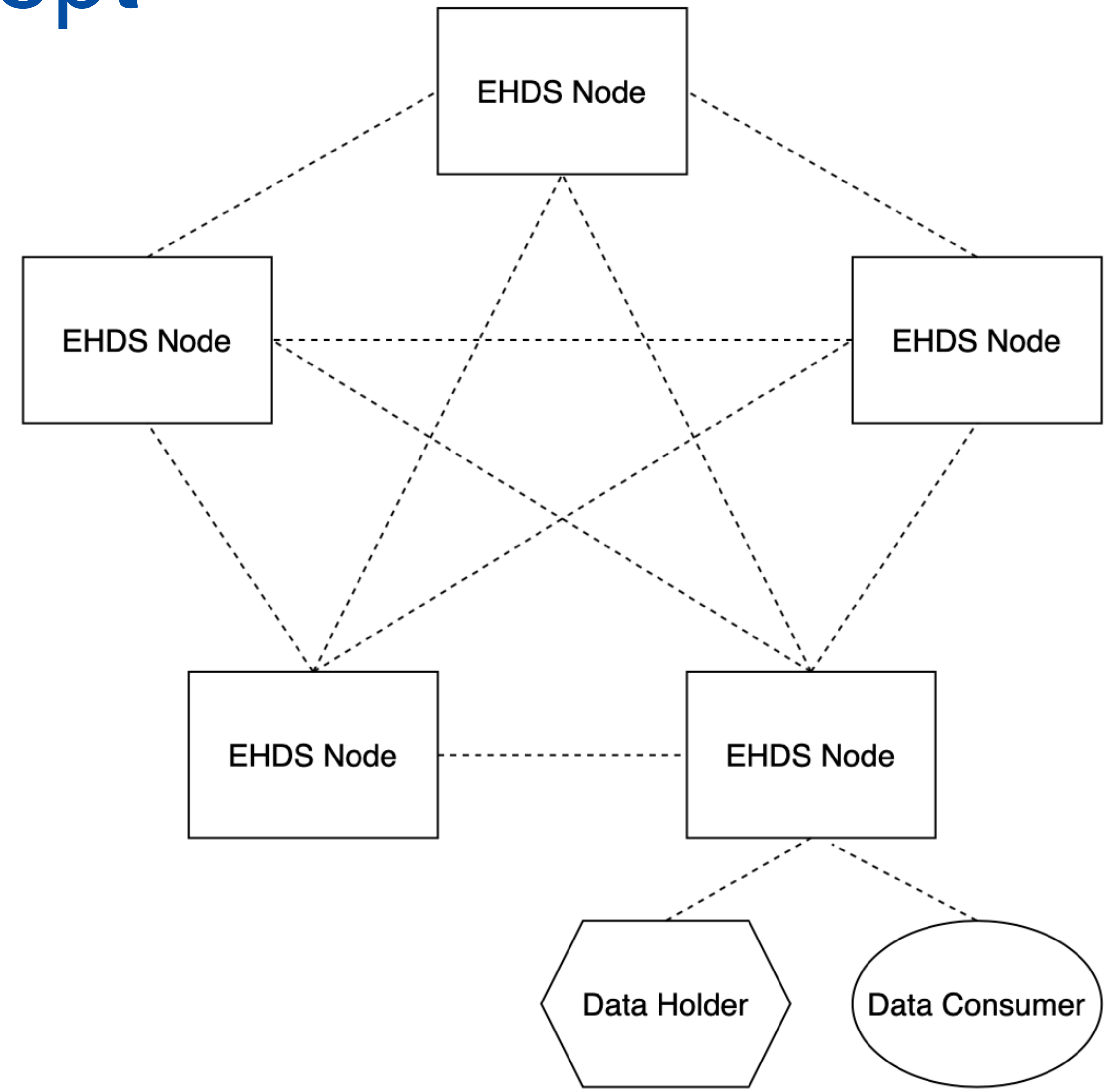
Demonstrator

Cross-country ecosystem

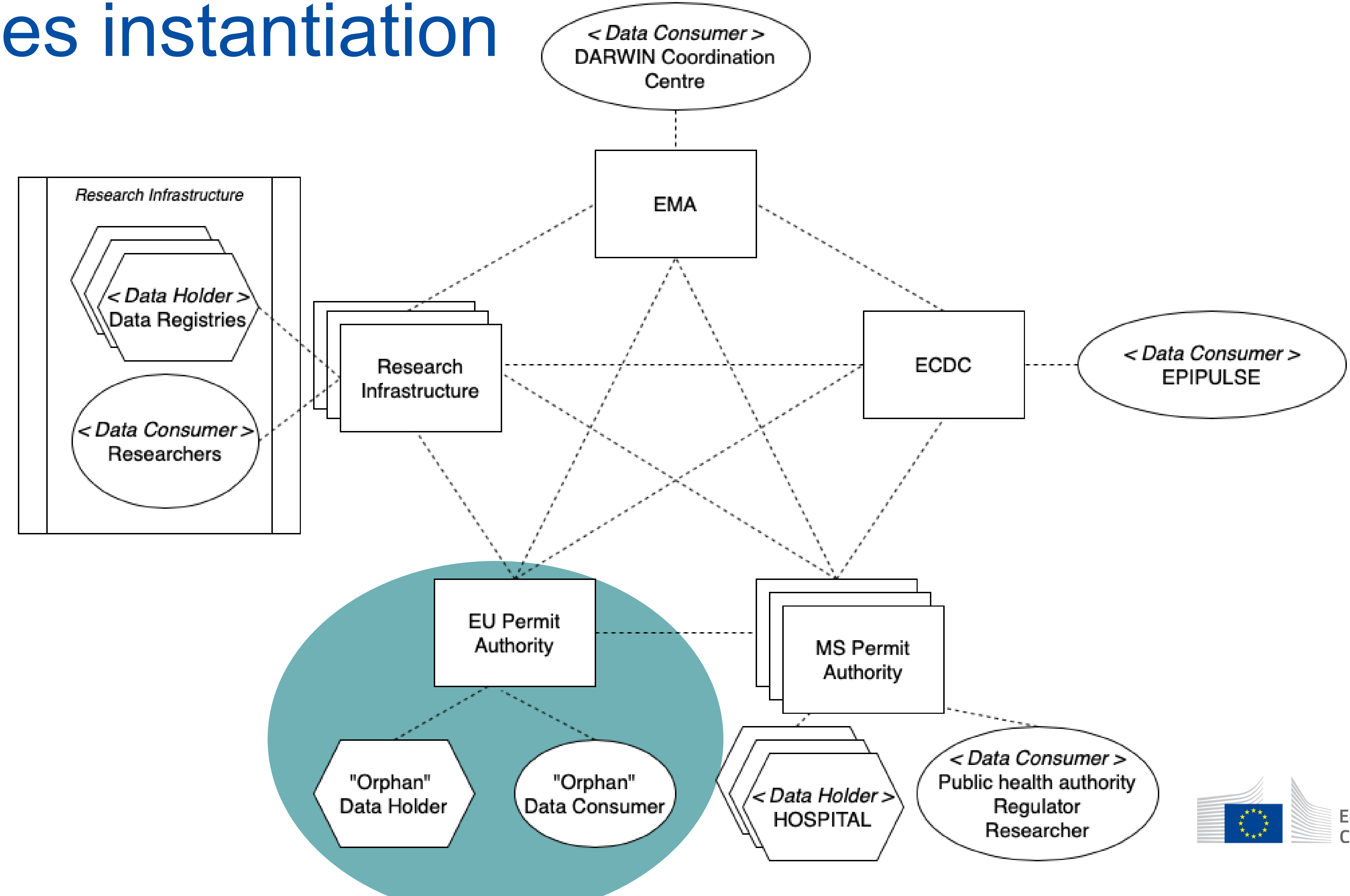
Secondary use of health data

EU4HEALTH

EHDS2: pilot: nodes concept



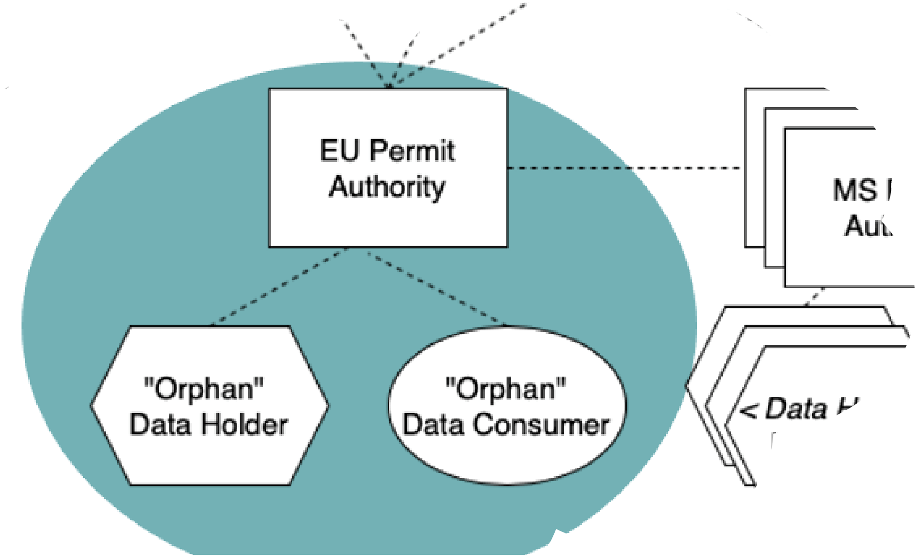
EHDS2: pilot: nodes instantiation



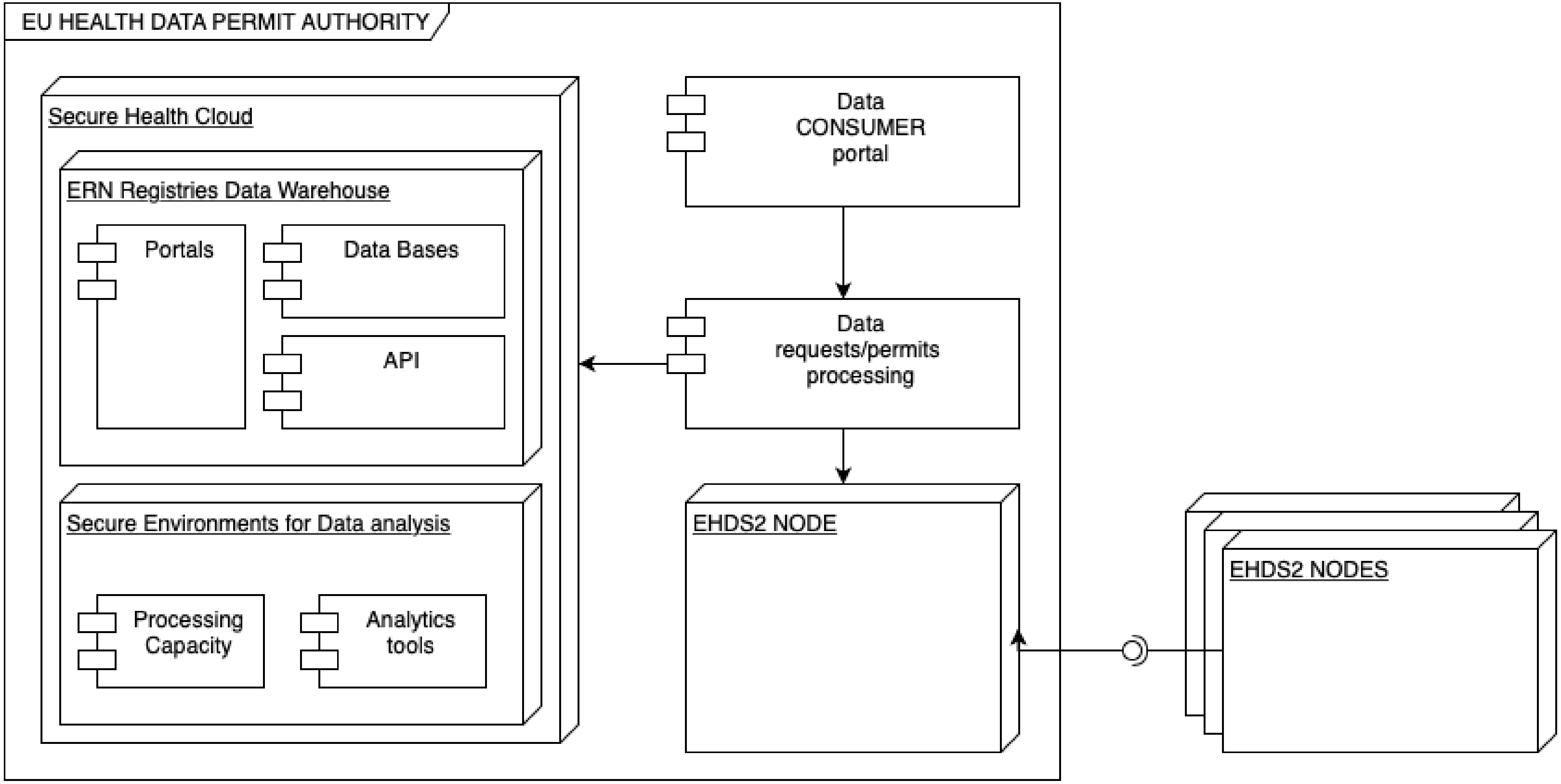
European Commission

EHDS2: EU Health Data Permit Authority

How can an EU Data Permit Authority support ERN registries towards interoperability and sustainability?



EHDS2: EU Data Permit Authority
candidate IT infrastructure architecture



EHDS2: EU Data Permit Authority
What's in there for me(Registries)?

Orchestrate permit requests (for ERN registries data)

Secure cloud (for ERN Registries solutions)

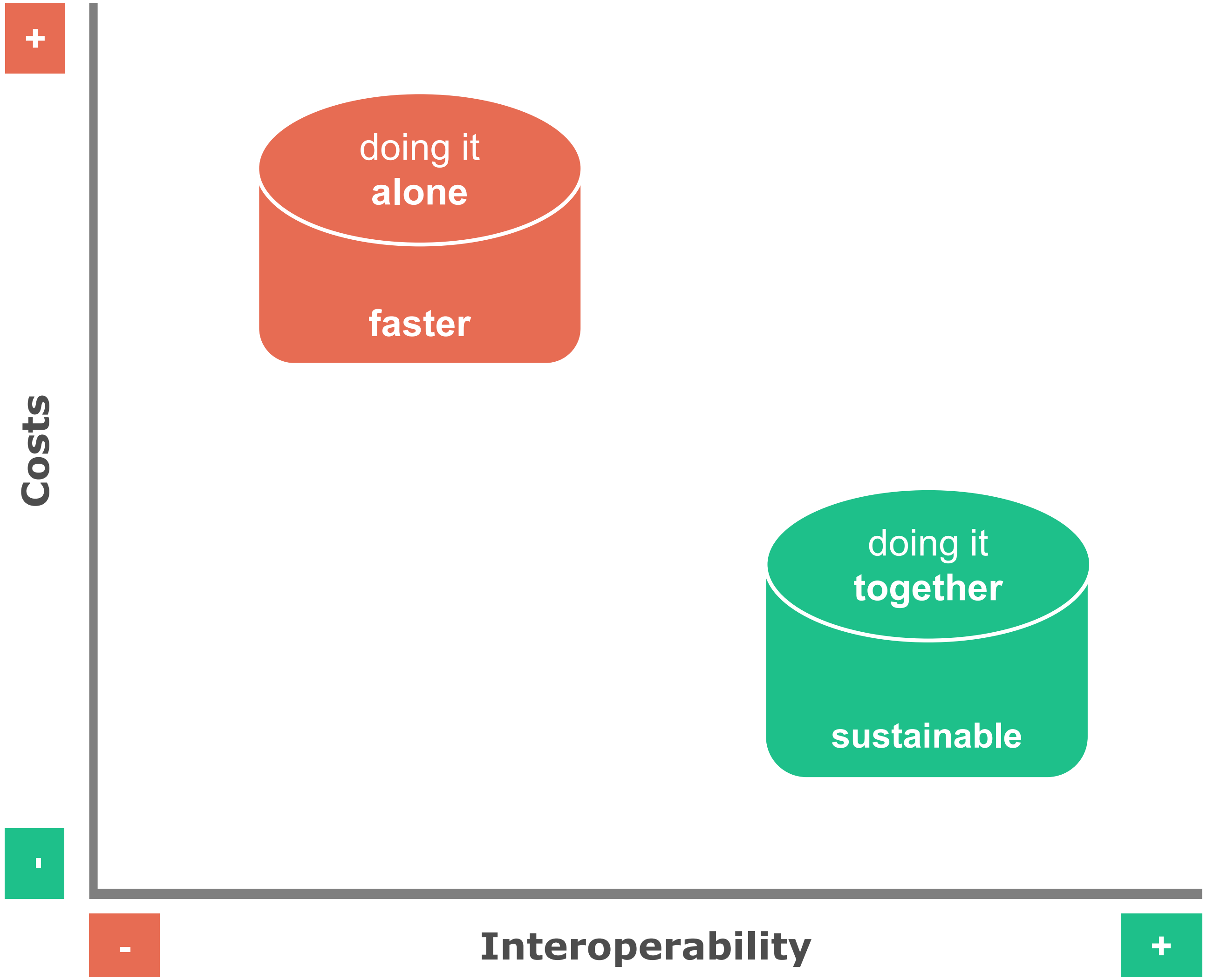
Reference Implementation(s) (ERN registries sw)

Tools for analytics and AI (secure inveronments and analytical tools)

IT solution provider (available to ERN registries organisations)

- **registry design, configuration and operation**
- **data migration and FAIRification services**

EHDS2: effort and accomplishment



Next steps

- We need to work together in further elaborating these proposals
  - Define clear requirements from ERN registries
  - Assess existing solutions and identify potencial to scale up
- Build a common and realistic time plan
- Align with upcoming EU4HEalth funding programme
- Build Capacity at ERN Registries level
- Orchestrate and Execute implementation plan

Questions?

Challenges?

Suggestions?

Further information

eHealth Network
https://ec.europa.eu/health/ehealth/policy/network_en

All events
https://ec.europa.eu/health/ehealth/events_en#anchor0

Any Other Business

Keep in touch

ERN overview:
https://ec.europa.eu/health/ern_en

ERNs and Covid19:
https://ec.europa.eu/health/ern/covid-19_en

ERN videos:
https://ec.europa.eu/health/ern/videos_en

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European
Reference
Networks

European
Commission

Thank you