



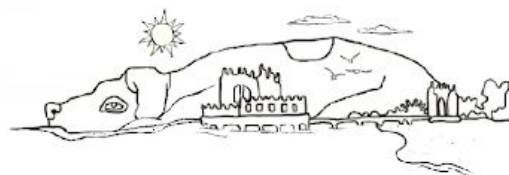
# SUMMER SCHOOL ProMIS

Edizione 3 – Anno 2023

**“Joint Actions in European Programming Period: a key tool for prioritising needs,  
improving capacity building and maximising implementation and impact in Regional  
Health Systems”**

**04 - 06 JULY 2023**

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Via Piano di Gallo, 11 – Mondello (Palermo)**



# Joint Action on integration of ERNs into National Healthcare Systems JARDIN

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# Outline

## Summary:

- Background
- General and specific objectives
- Complementarity with other actions

Consortium set-up

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Work plan & Work packages

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# Background

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More than **6,000 rare diseases** (RD) are known to date, with an estimate of 30 million people living with a RD in Europe [1], making effective measures for diagnosis and treatment a necessity. RD patients are typically scattered across countries, rendering the acquisition of knowledge and experience difficult. Therefore, centralization is key for RD, with regard to expertise, as well as equipment and infrastructure.

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The EU recognized and acknowledged this fact and developed the **European Reference Networks (ERN)** model as a coordinated cooperation between all member states (MS) in the field of highly specialized health services, thereby generating immense added value for European citizens.

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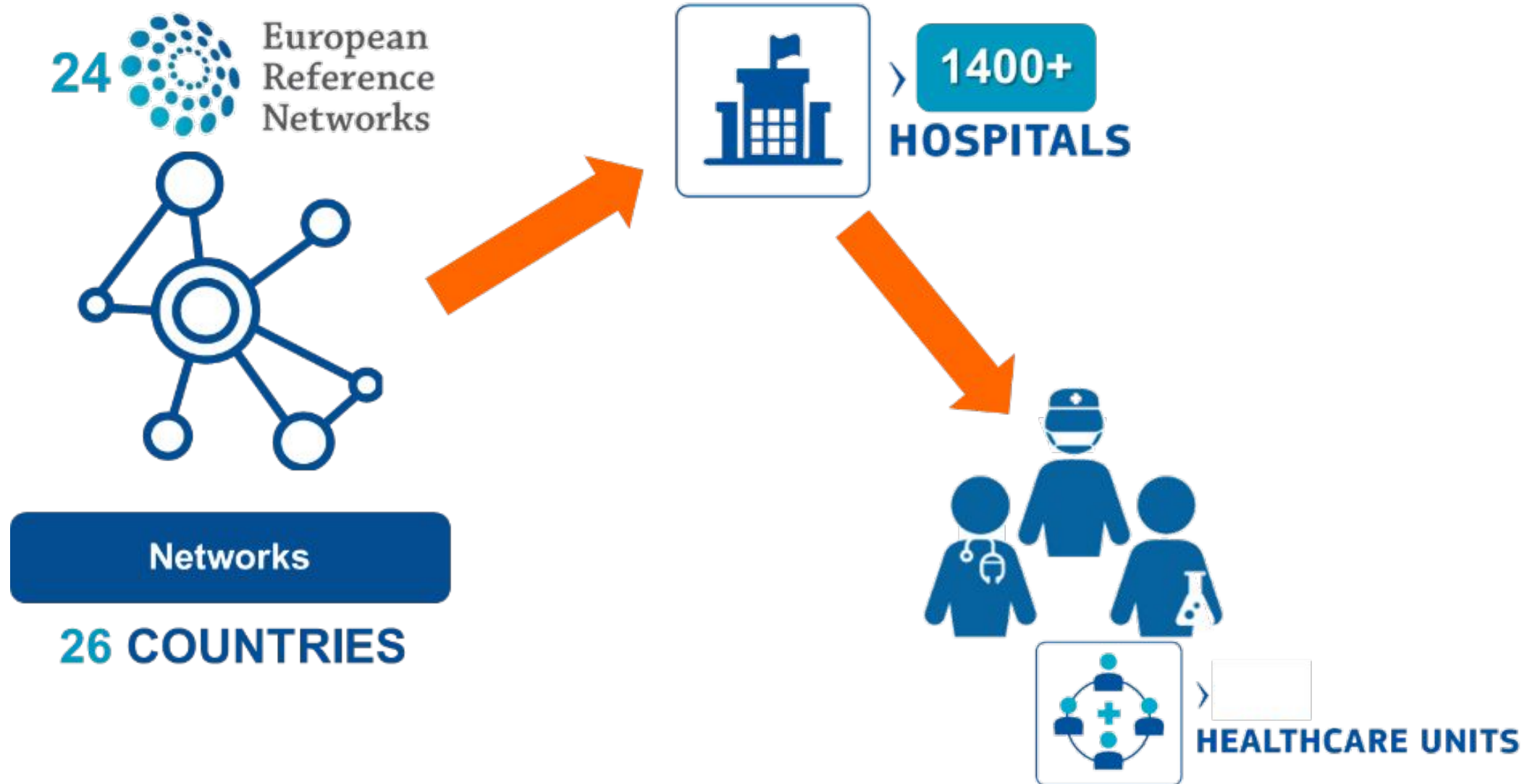
24 ERN established in March 2017 are **multi-national networks of highly specialized healthcare providers (HCP)** across Europe organized in thematically coherent medical domains, to improve patients' access to high-quality healthcare as well as support knowledge generation, professional training and education, and research.

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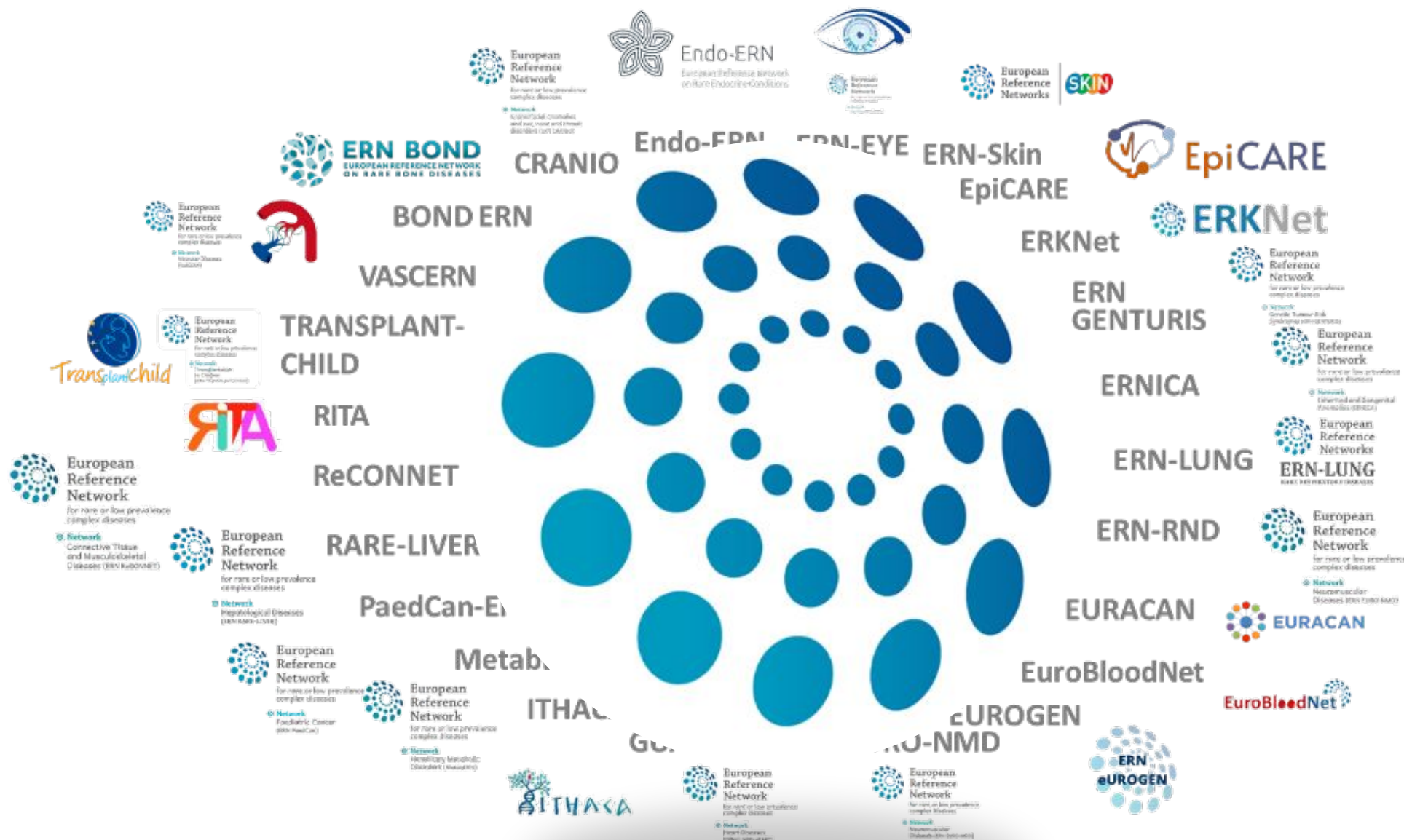
# European Reference Networks - (ERNs)

**ERNs** are **virtual networks** of European centres of expertise, formally accredited by their respective Member State, created with **the aim to join the best specialists from across Europe to tackle complex or rare medical** conditions that require highly specialised healthcare and a concentration of knowledge and resources.

# ERNs' indices

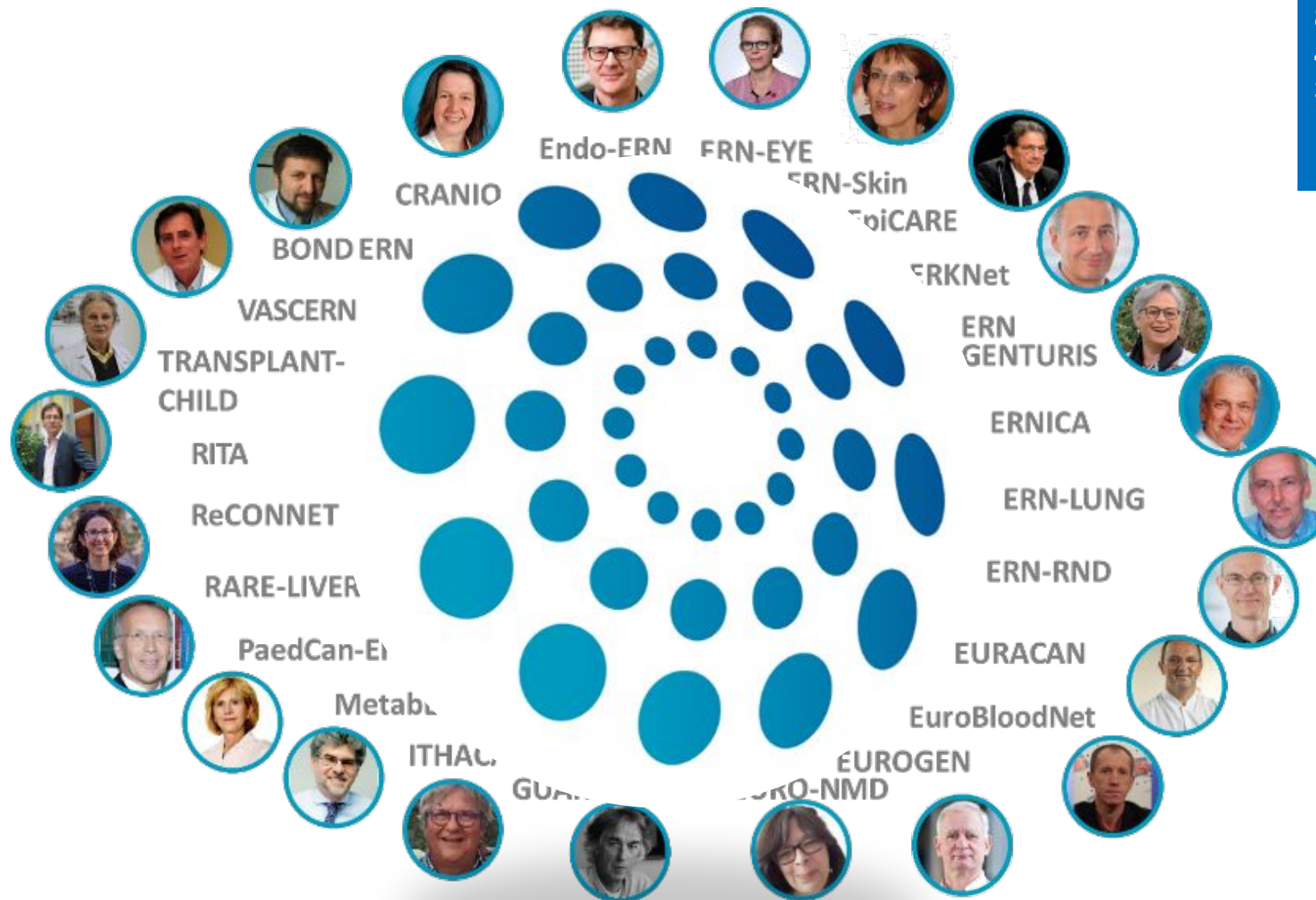


# ERNs: 24 thematic networks





# ERN Coordinators



7 Netherlands  
8 France  
4 Germany  
3 Italy  
1 Austria  
1 Spain



# General and specific objectives

MS should be encouraged to integrate the ERNs into their healthcare systems with the overall objective

- to facilitate access to timely diagnosis and high-quality and cost-effective healthcare for all patients with rare or low-prevalence complex diseases.

This Joint action (JA) aims:

- to improve the accessibility and support the long-term sustainability of the ERN system by contributing to the effective integration of ERN in the national health systems
- strengthening the resilience of the national health systems.

Activities will include:

- identification and exchange of best practices
- development of concrete recommendations, guidelines, and toolboxes suited for the needs of all MS
- support of capacity building and performing pilot implementation steps on different levels in MS in the proposed fields of action.

# Complementarity with other actions

**EHDS Pilot 2**

**European Joint  
Programme on Rare  
Diseases (EJP RD,  
2019-2023)**

**The European Rare  
Disease Registry  
Infrastructure (ERDRI)**

**European Rare Disease  
Research Coordination  
and Support Action  
(ERICA, 2021-2025)**

**IRDiRC (International Rare  
Diseases Research  
Consortium)**

**OD4RD and OD4RD2  
(2022-2023, 2023-2025)**

**Towards the European  
Health Data Space  
(TEHDAS, 2021-2024)**

**Solve-RD (2018-2023)**

**X-eHealth (2020-2022)**

# Consortium set-up

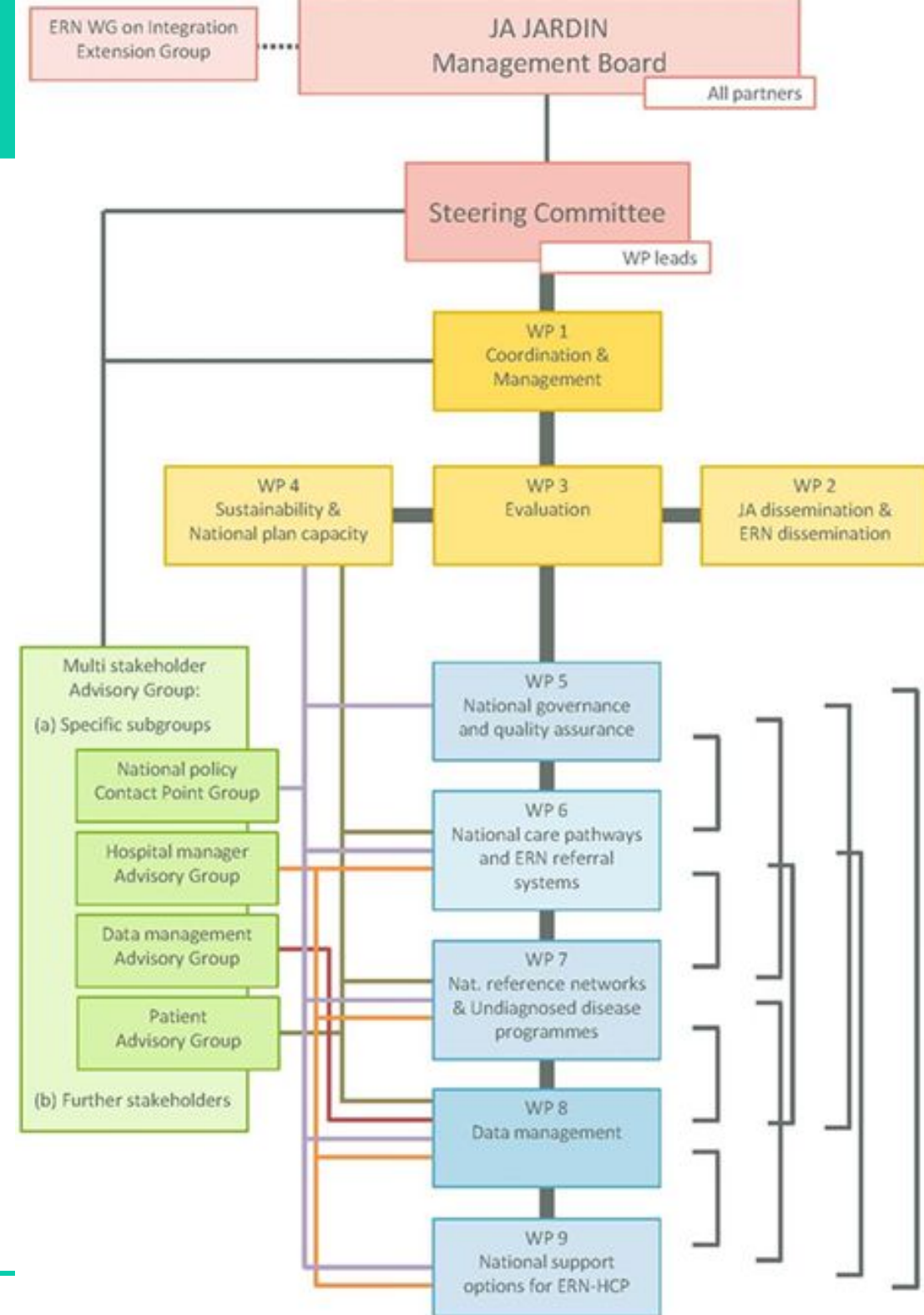
Austria - Medical University of Vienna (MUW; CA)	Belgium - Federal Public Service Health, Food Chain Safety and Environment (FPS HFCSE; CA)	Belgium - Sciensano (AE)	Bulgaria - Medical University - Sofia (MUS; CA)	Cyprus - Ministry of Health of Cyprus (MoH CY; CA)	Czech Republic - General University Hospital in Prague (GUH; CA)	Germany - Bundesministerium für Gesundheit (BMG; CA)	Germany - The Federal Institute for Drugs and Medical Devices (BfArM; AE)	Germany - The National Action League for People with Rare Diseases (NAMSE; AE)
Germany - The University Hospital Tübingen (UKT; AE)	Germany - Heidelberg University Hospital (UKHD; AE)	Germany - The University Hospital Würzburg (UKW; AE)	Germany - Universitätsklinikum Frankfurt (UK FFM; AE)	Denmark - The University Hospital Aarhus (AUH; CA)	Estonia - Tartu University Hospital (TUH; CA)	Greece - Ethnikos Organismos Dimosias Ygeias (EODY; CA)	Greece - Medical School of National and Kapodistrian University of Athens (NKUA; AE)	Spain - Fundación Para La Investigación Biomedica Del Hospital Universitarios La Paz (FIBHULP; CA)
Spain - Health Research Institute Hospital La Fe (HULAFE; AE)	Finland - Finnish Institute for Health and Welfare (THL; CA)	France - Ministère de la santé (DGOS; CA)	France - Hôpitaux Universitaires de Strasbourg (HUS; AE)	France - Hospices Civils de Lyon (HCL; AE)	France - Assistance Publique-Hôpitaux de Paris (APHP; AE)	France - Institut National de la Santé et de la Recherche Médicale (INSERM; AE)	Croatia - University Hospital Center Zagreb (UHCZ; CA)	Croatia - The Ministry of Health of the Republic of Croatia (MoH-HR; AE)
Hungary - The University of Debrecen (UD; CA)	Hungary - The Bethesda Children's Hospital (BCH; AE)	Hungary - Semmelweis University (SU; AE)	Ireland - Health Service Executive (HSE; CA)	Italy - Istituto Ortopedico Rizzoli (IRCCS IOR; CA)	Italy - Azienda Ospedaliero Universitaria Pisana (AOUP; AE)	Italy - Istituto Superiore di Sanità (ISS; AE)	Italy - IRCCS Ospedale Policlinico San Martino (HSM; AE)	Italy - Veneto Region (AE)
Italy - Azienda Sanitaria Univeritaria Friuli Centrale (ASUFC; AE)	Italy - Fondazione Policlinico Universitario Agostino Gemelli IRCCS (FPG; AE)	Italy - The Italian Ministry of Health (IT-MoH)	Lithuania - Lithuania Vilnius University Hospital Santaros Klinikos (VULSK; CA)	Luxemburg - The Direction de la Santé (DISA; CA)	Latvia - Children's Clinical University Hospital (CCUH; CA)	Malta - Ministry for Health-Government of Malta (MFH; CA)	The Netherlands - The Ministry of Health, Welfare and Sports (VWS; CA)	The Netherlands - The Radboud University Medical Center (RUMC; AE)
The Netherlands - Leiden University Medical Centre (LUMC; AE)	The Netherlands - Erasmus Medical Center Rotterdam (Erasmus MC; AE)	Norway - South Eastern Norway Regional Health Authority (HSO; CA)	Norway - Norwegian Directorate of Health (HDIR; AE)	Norway - Oslo University Hospital (OUS; AE)	Poland - Ministry of Health Poland (MoH PI; CA)	Portugal - Ministério da Saúde – República Portuguesa (DGS; CA)	Portugal - Centro Hospitalar de Lisboa Norte (CHLN; AE)	Romania - Emergency Clinical County Hospital Craiova (ECCHC; CA)
	Sweden - The National Board of Health and Welfare (SoS; CA)	Slovenia - University Medical Center Ljubljana (UMCL; CA)	Slovakia - Ministry of Health of the Slovak Republic (MoH SR; CA)	Slovakia - The National Institute for Children's diseases (NICHD; AE)	Ukraine - Ministry of Health of Ukraine (AP)	Switzerland - Les Hôpitaux Universitaires de Genève (HUG; AP)		

# Consortium set-up

Italy 8	Belgium 2	Austria 1	Finland 1	Poland 1
Germany 7	Croatia 2	Bulgaria 1	Ireland 1	Romania 1
France 5	Greece 2	Cyprus 1	Lithuania 1	Sweden 1
Hungary 3	Portugal 2	Czech Republic 1	Luxemburg 1	Slovenia 1
The Netherlands 4	Slovakia 2	Denmark 1	Latvia 1	Ukraine 1
Norway 3	Spain 2	Estonia 1	Malta 1	Switzerland 1

# Consortium structure

Core of the Consortium are the four horizontal work packages (WP) (orange group) and the five vertical WPs (blue group) which are tightly connected by regular, as well as ad hoc virtual meetings on the lead level (thick grey cross), configurational represented in the Steering Committee (SC) (red group).



# Workplan and workpackages

- The activities of this project are structured in 9 work packages (WP):
- 4 transversal ones dedicated to the coordination, dissemination, evaluation, and sustainability of the JA
- 5 technical ones aiming to cover all aspects of the integration of ERN into national health systems



# WP1: Project management and coordination

Duration: M1 – M36

Lead Beneficiary: MUW (Medical University of Wien)

## Objectives

- Establish an effective and efficient governance,
- Monitor and guide the activities and ensure the quality of the JA implementation,
- Ensure effective communication and information exchange among the JA partners and governance and advisory bodies,
- Provide day to day administrative support to the partners,
- Ensure appropriate financial administration with regard to budgeting, planning, and accounting,
- Ensure all communication with HaDEA and DG SANTE, including timely presentation of all deliverables and technical and financial reports,
- Ensure appropriate risk management

# WP2: Dissemination

Duration: M1 – M36

Lead Beneficiary: FIBHULP (Fundación Para La Investigación Biomedica Del Hospital Universitarios La Paz)

Objectives:

- To achieve efficient and effective visibility, awareness, and acceptance of the JA to internal and external stakeholders
- To support national ERN-specific dissemination activities
- To develop a blueprint for national dissemination strategies on ERNs

## WP2: Dissemination

The main communication language of the JA will be English. Translation can be offered for the 24 official EU languages and Norwegian. The definition of the audience needs will be carried out by:

- segmenting the audience into levels of interest and influence in readiness for development of channel strategy and key messages. Stakeholders will cover the target groups, especially primary care clinicians and patients. In person or online national meetings will be conducted;
- holding communications workshops/meetings with other WP, MS, and ERNs communications leads to understand their specific needs and challenges

## WP3: Evaluation

Duration: M1 – M36

Lead Beneficiary: UHCZ (University Hospital Center Zagreb)

### Objectives

- To verify if the project is being implemented as planned and reaches the objectives and to evaluate whether the JA as a whole has produced planned results, delivered expected benefits, and made the desired change (evaluation of process, outputs, and outcomes).

Other objectives include:

- to evaluate if project processes are going according to plan;
- to evaluate whether the participants (WP leaders, stakeholders, MS representatives, etc.) are satisfied with the project processes;
- to assess the outcomes of the JA;
- to monitor whether deliverables are produced on time and following the proposed objectives;
- to evaluate the ability to implement the new findings of th JA in MS

## WP4: Sustainability of outcomes and outputs of the Joint Action

Duration: M1 – M36

Lead Beneficiary: VULSK (Lithuania Vilnius University Hospital Santaros Klinikos)

Objectives:

To develop the JA sustainability strategy including:

- sustainability of JA actions at MS level and
- mechanism for sustainability/accountability at the EU level
- support capacity building in MS for the elaboration of new/updated National Plans/Strategies for RD (in terms of sustainability of JA actions).

## WP4: Sustainability of outcomes and outputs of the Joint Action

A strategy for the sustainability of JA actions will include measures at European and national level that are continuous, i.e., both precede and will be continued after the accomplishment of JA (ERN BoMS and its Working Group on ERN integration into national systems, and ERNs) and those that will be purposefully developed for this JA (National Policy Contact Point group).



## WP5: National governance and quality assurance models

Duration: M1 – M36

Lead Beneficiary: IOR (Istituto Ortopedico Rizzoli); co-leads MUW, AOUP

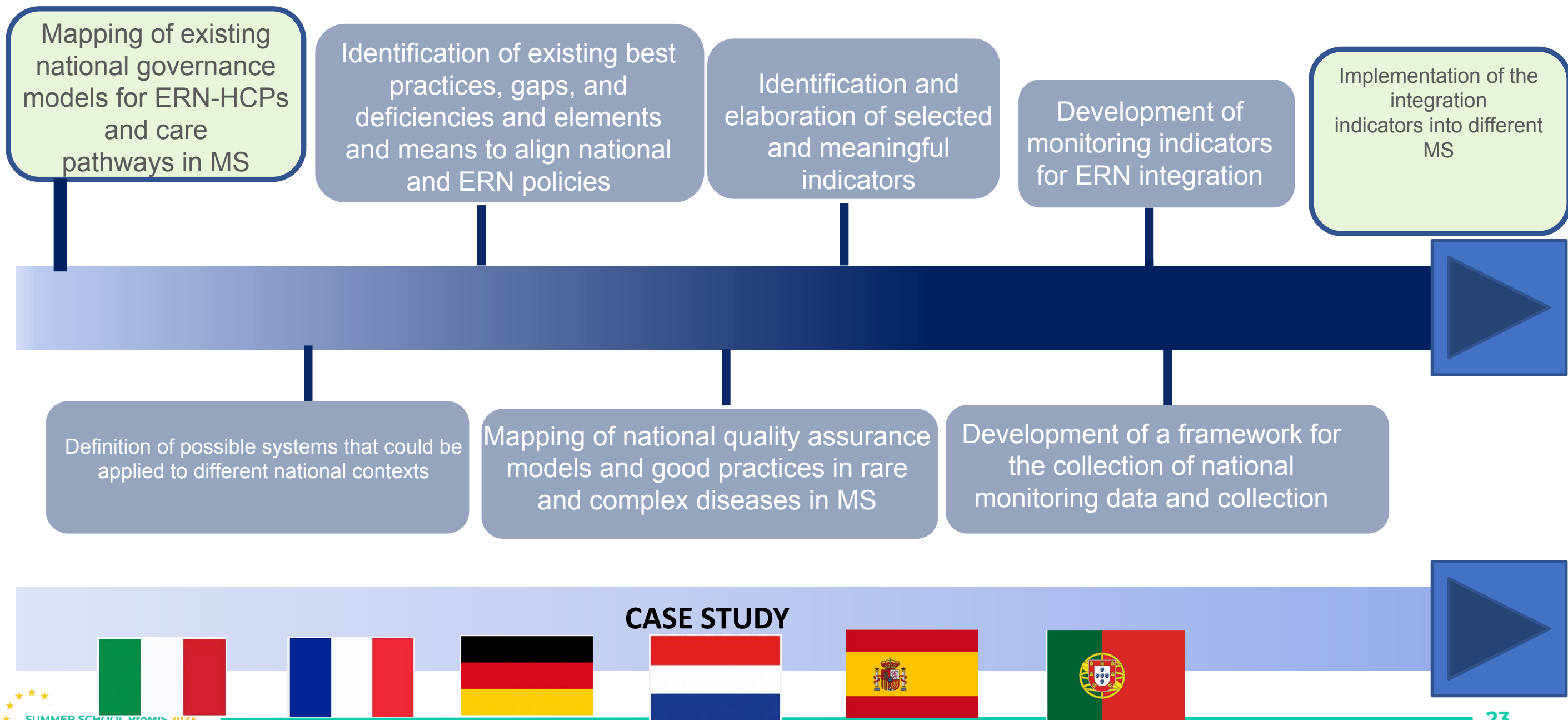
Objectives:

- To develop proposals for national governance models and practices for rare and complex disease HCPs and care pathways, fully interoperable with ERNs
- To develop a proposal for national quality assurance models for rare and complex diseases

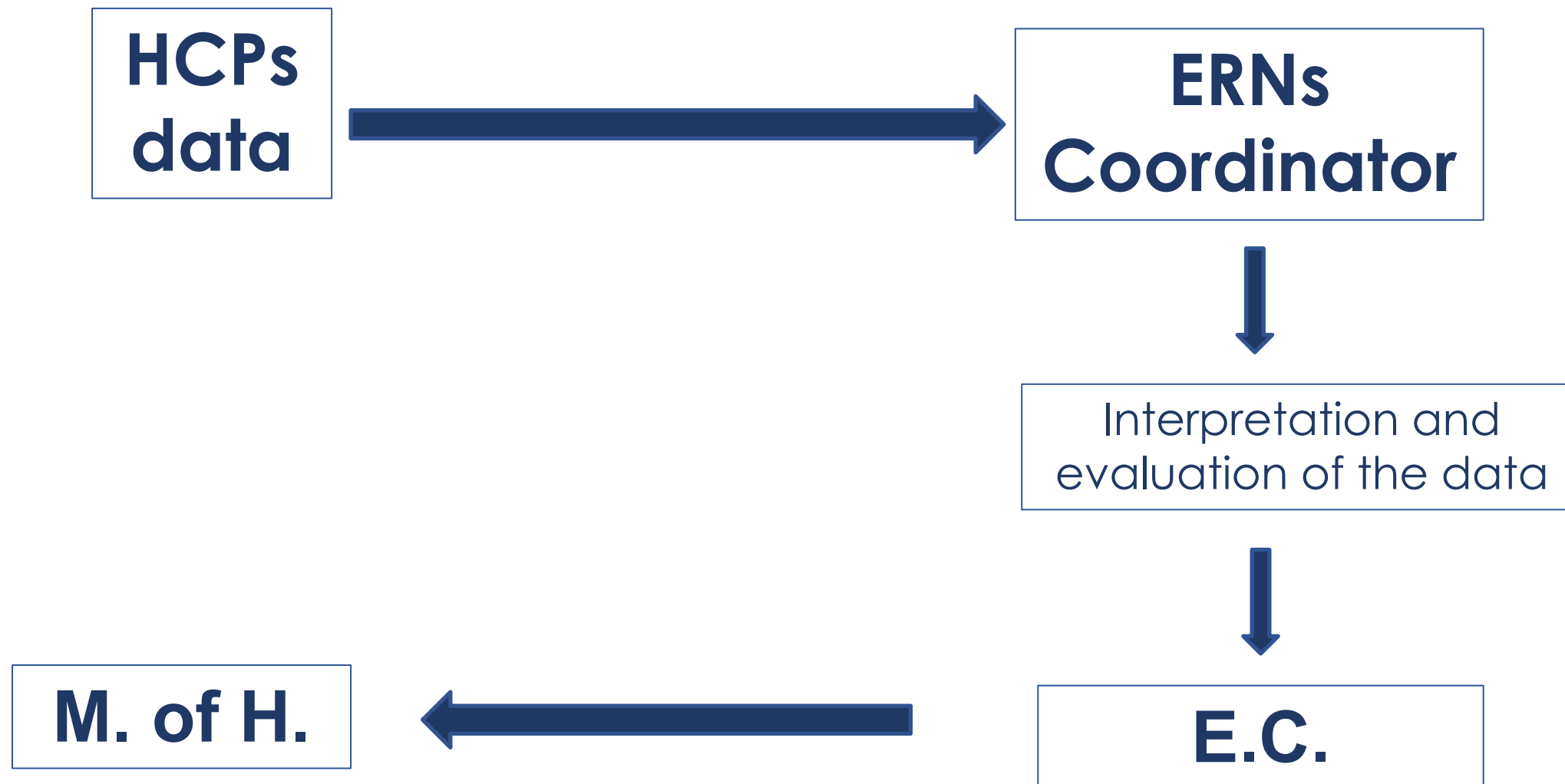
## WP5: National governance and quality assurance models

In this WP we will explore the current situation in the different MS through survey and mapping of existing national governance models for ERN-HCPs and care pathways; proceed to the identification of existing best practices, gaps, and deficiencies. evaluate all elements and means to align national and European ERN policies; and elaborate possible strategies and models for the integration of ERNs into the different types of national healthcare systems.

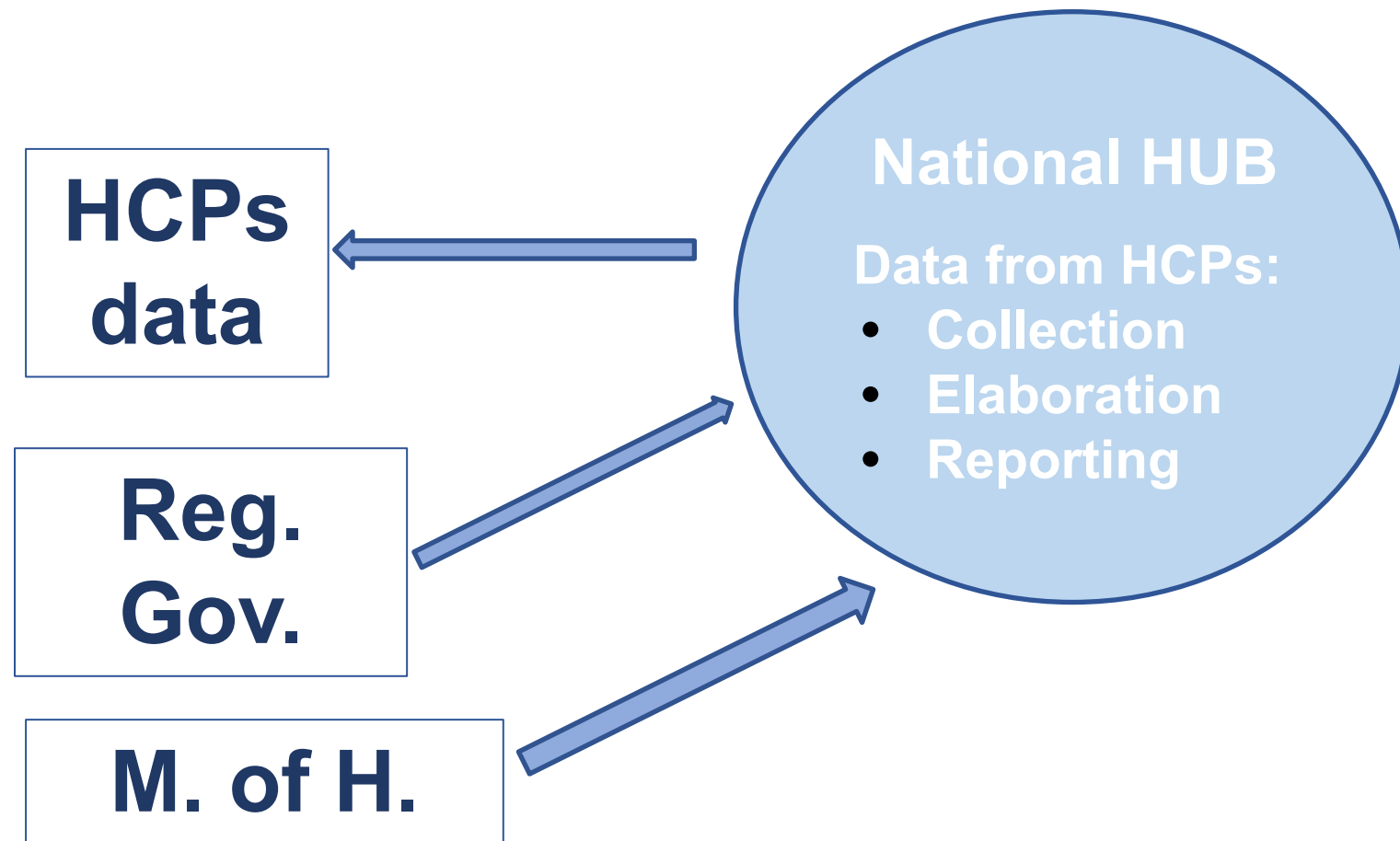
In order to develop national indicators aimed at mapping the level of integration of the ERNs into the national healthcare systems in Europe, a co-designed approach will be adopted.



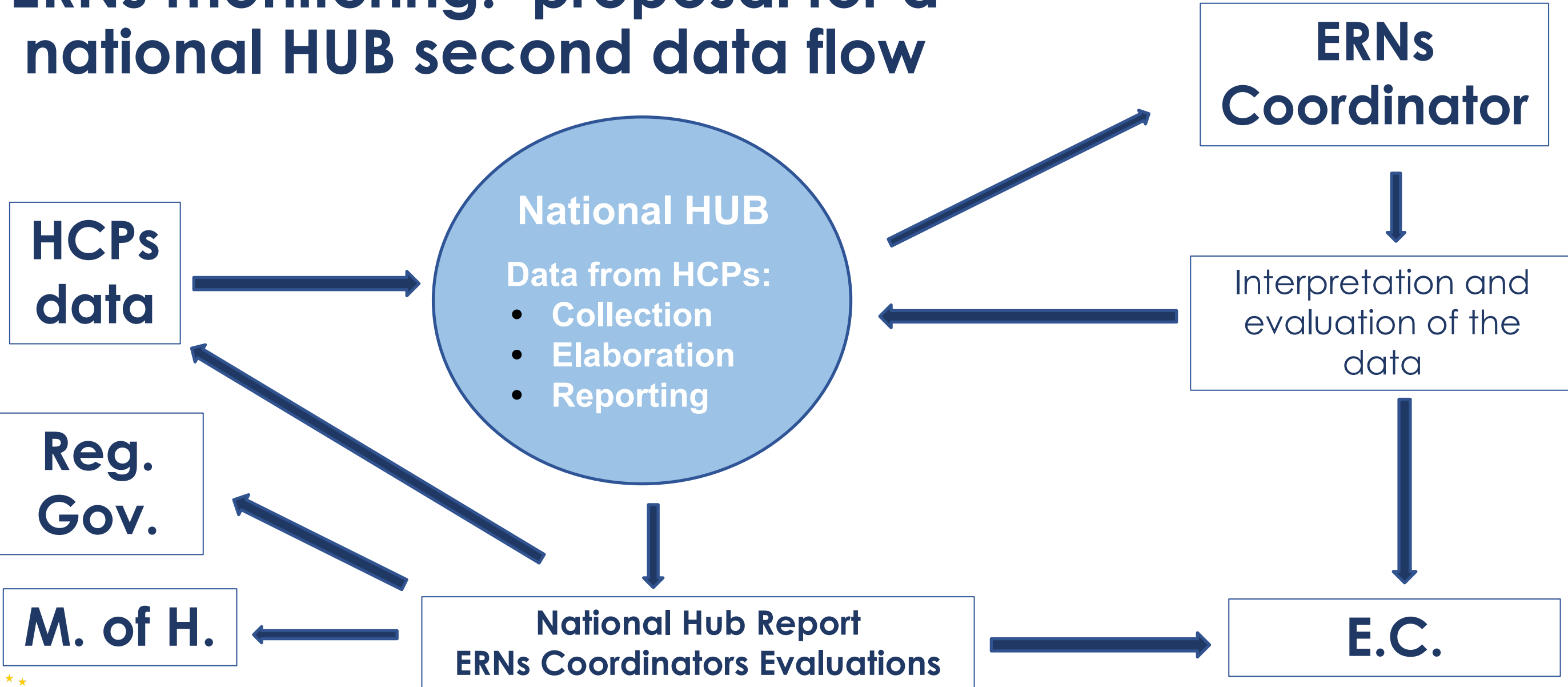
# ERNs monitoring: current data flow



# ERNs monitoring: proposal for a National HUB starting data flow

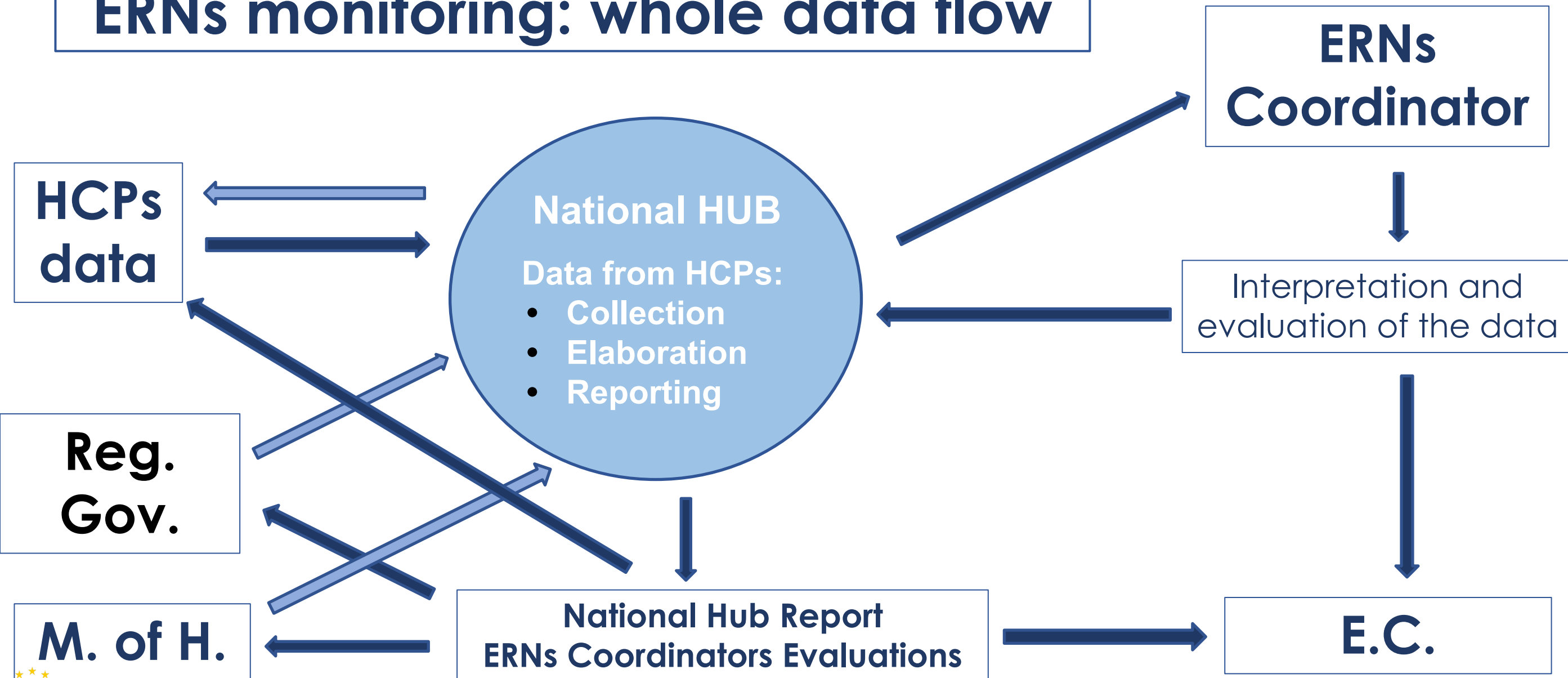


# ERNs monitoring: proposal for a national HUB second data flow





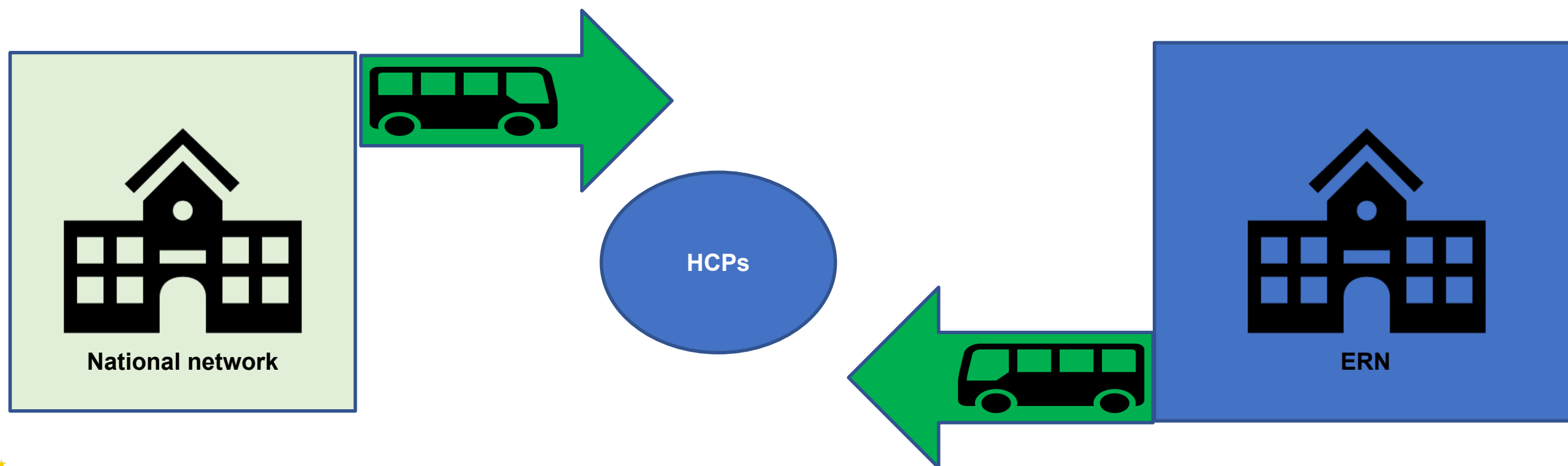
# ERNs monitoring: whole data flow



# Network HCP levels

- **National HCP**
- **ERN HCP**

Following the evaluation period each HCP can be identified such as eligible or not for the inclusion in the ERNs in order to have a two level dynamic network.



# WP6: National care pathways and ERN referral systems

Duration: M1 – M36

Lead Beneficiary: VULSK; co-leads HSE, MUW

## Objectives:

- To develop recommendations for the organization of national care pathways for rare and complex diseases interfacing with ERNs, including the recognition of and preferably full compliance with ERN-elaborated evidence-based resources (like Clinical Practice Guidelines),
- To develop a proposal for referral systems to ERNs;
- To develop guidelines for the incorporation of CPMS advice into patients' care

## WP6: National care pathways and ERN referral systems

This will include a complete cycle from development of a sign-posting tool and model reference care pathways for identified RD or groups of RD to the comprehensive analysis of barriers and enablers for their implementation, to real-life pilots of care pathways in national systems and to the development of recommendations for care pathways, case management / care coordination, referrals to ERNs and inclusion of CPMS advice, based on the diversity of MS and set for continuous improvement.

In addition, we will develop recommendations for an early and continued emergency response to sustain healthcare support and care pathways in nationwide disaster situations with severe impairment of the regular healthcare system.

## **WP7: National reference networks and undiagnosed disease programmes (UDP) or equivalent strategies interlinked with ERN**

Duration: M1 – M36

Lead Beneficiary: UKT (The University Hospital Tübingen); co-lead MUW

### Objectives

- To support capacity building in MS for the development of NRN or equivalent strategies for rare and complex diseases and their integration with ERN
- To develop structures and procedures for undiagnosed patients closely linked to ERN on a national and European level

## **WP7: National reference networks and undiagnosed disease programmes (UDP) or equivalent strategies interlinked with ERN**

For the first objective, three tasks will be performed: a state-of-the-art analysis of existing structures, models and initiatives on member state level will be performed; a comprehensive survey will collect overview information from the member states. Models and recommendations for national reference networks for rare and complex diseases and their integration with ERNs will be developed by means of a multi-stakeholder consensus process. Then, capacity building workshops and pilot implementation for national reference networks, or equivalent strategies, will be done.

For the second objective, we will first perform a state-of-play analysis and, based on the results, develop recommendations for national UDP or equivalent strategies, including their national and international integration and national governance.



# WP8: Data management

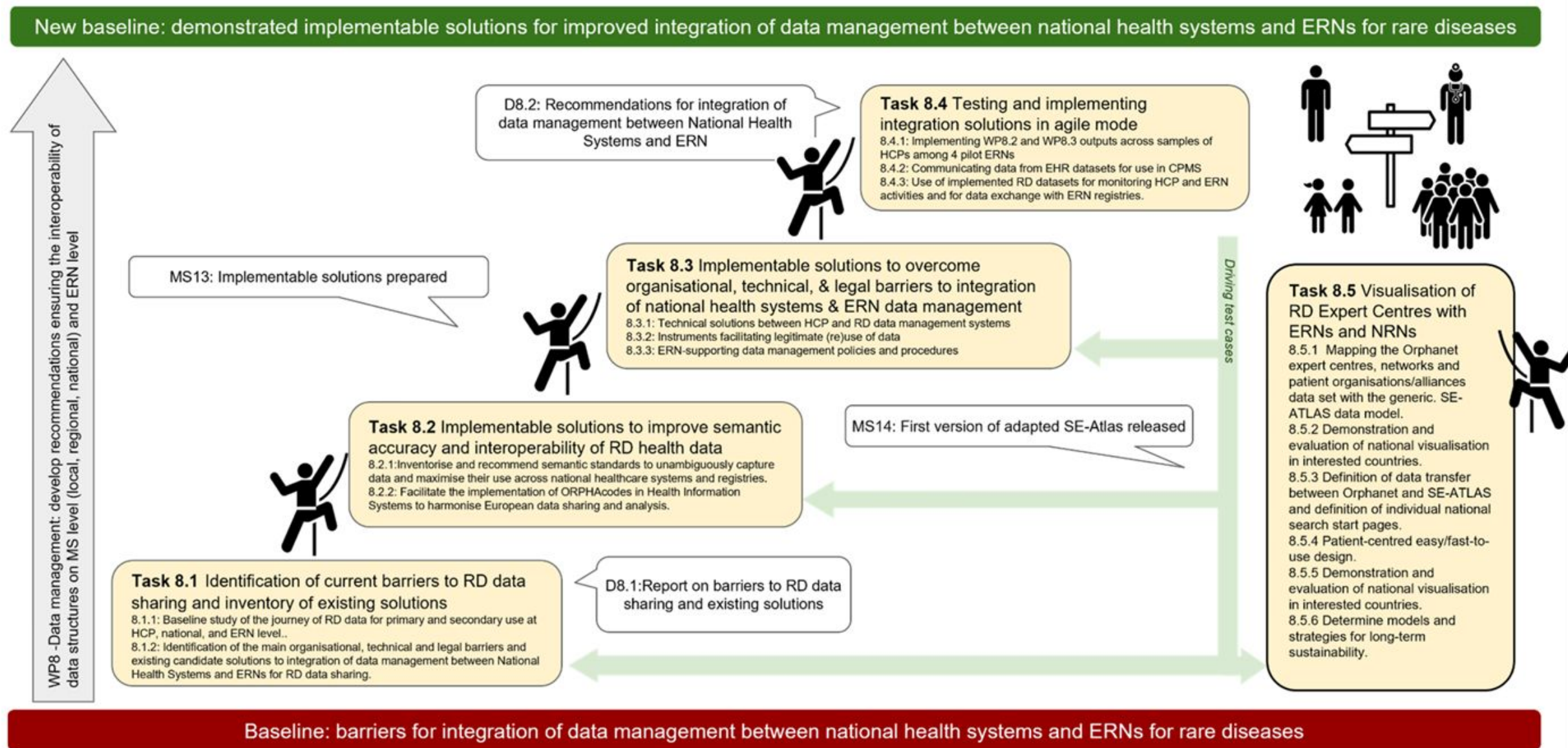
Duration: M1 – M36

Lead Beneficiary: DGOS (Ministère de la santé); co-lead VWS

Objectives

- To develop recommendations ensuring the interoperability of data structures on MS level (local, regional, national) and ERN level.

# WP8 Tasks



## WP9: National support options for ERN-HCP

Duration: M1 – M36

Lead Beneficiary: GUH (General University Hospital in Prague)

### Objectives

- To collect and analyse good practices and mechanisms to provide support to ERN-hosting healthcare providers at national level as well as to individual ERN centres at the hospital (HCP) level
- To develop specific recommendations for: 1. the national support to healthcare providers participating in ERNs, 2. the hospital support to individual ERN centres, 3. the CPMS service and reimbursement models

## WP9: National support options for ERN-HCP

WP9 will map the situation and define the main barriers of various types (e.g. organisation, funding) at various levels (hospital - regional - national) across ERNs and member states. Series of online surveys will be directed at selected ERN clinical centres and defined groups of stakeholders (e.g. national authorities, payers, charities, patient advocacy groups) to address multiple potential mechanisms of cooperation and support.

# Conclusions

- There is currently no guidance for MS how to ensure high quality, safe and integrated care for persons living with a RD.
- This JA will lay the foundation for a mature, all-encompassing ERN ecosystem fulfilling the overall vision of ERNs “to provide the framework for healthcare pathways [...] through a high level of integrated expertise”, and eventually serve all patients with rare and low prevalence complex diseases, “including those in the process of seeking a diagnosis or in whom a final diagnosis is not yet confirmed”
- In the long term, access to timely and accurate diagnosis, provision of high quality, safe and integrated care, health indicators, health-related quality of life of patients, and sustainability of health systems will be improved.



