EuroBloodNet

European Reference Network in Rare Hematological Diseases

Cross Border Health: ERN-EuroBloodNet's overview for reaching equal access to care across EU MS

Mariangela Pellegrini
ERN Manager
on behalf of ERN-EuroBloodNet Coordination Team

Economical perspectives in Rare Diseases.
9th ECRD Conference 10-12 May 2018

European Reference Networks

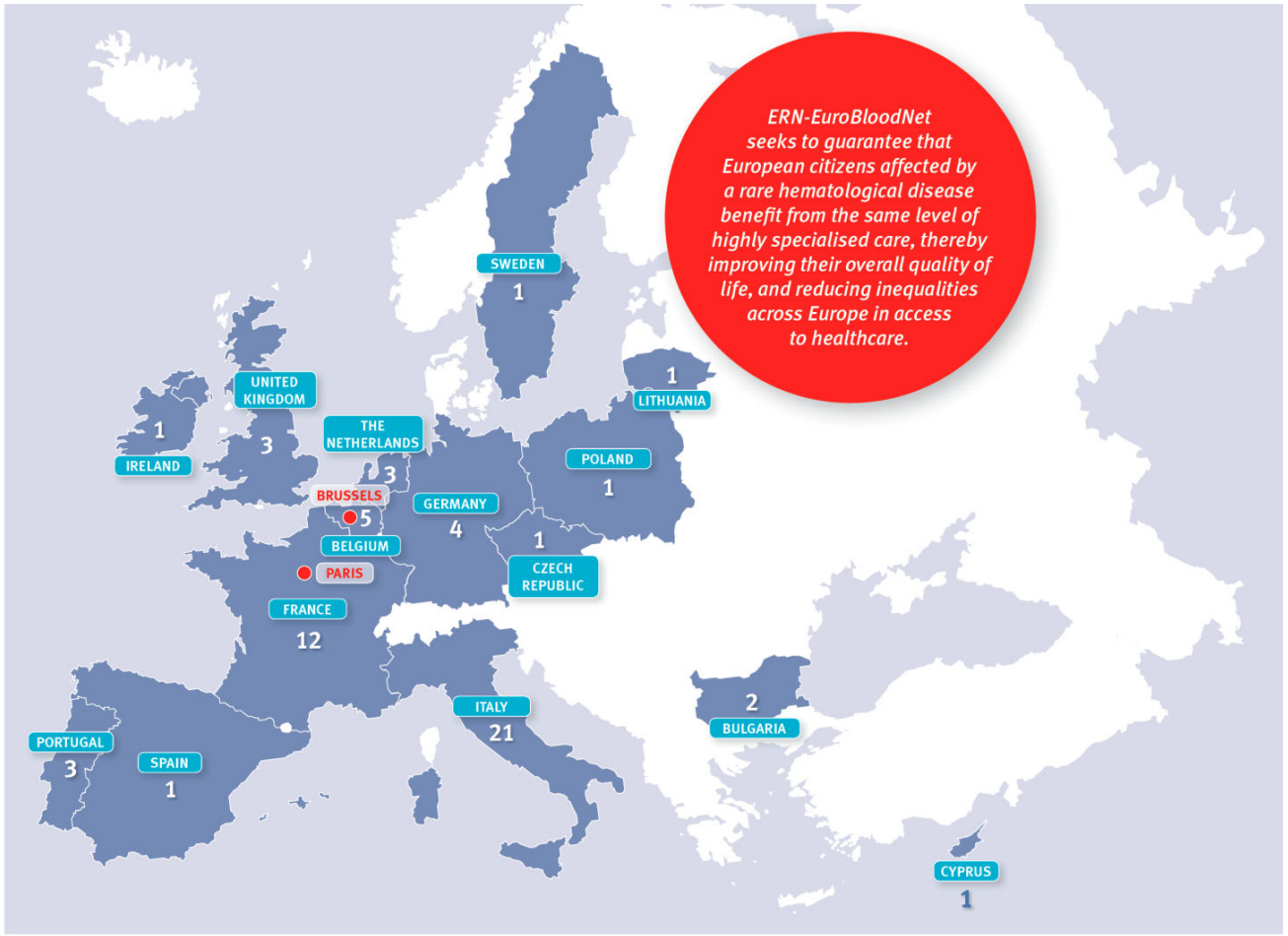
European Commission

Co-funded by the Health Programme of the European Union

ERN-EuroBloodNet

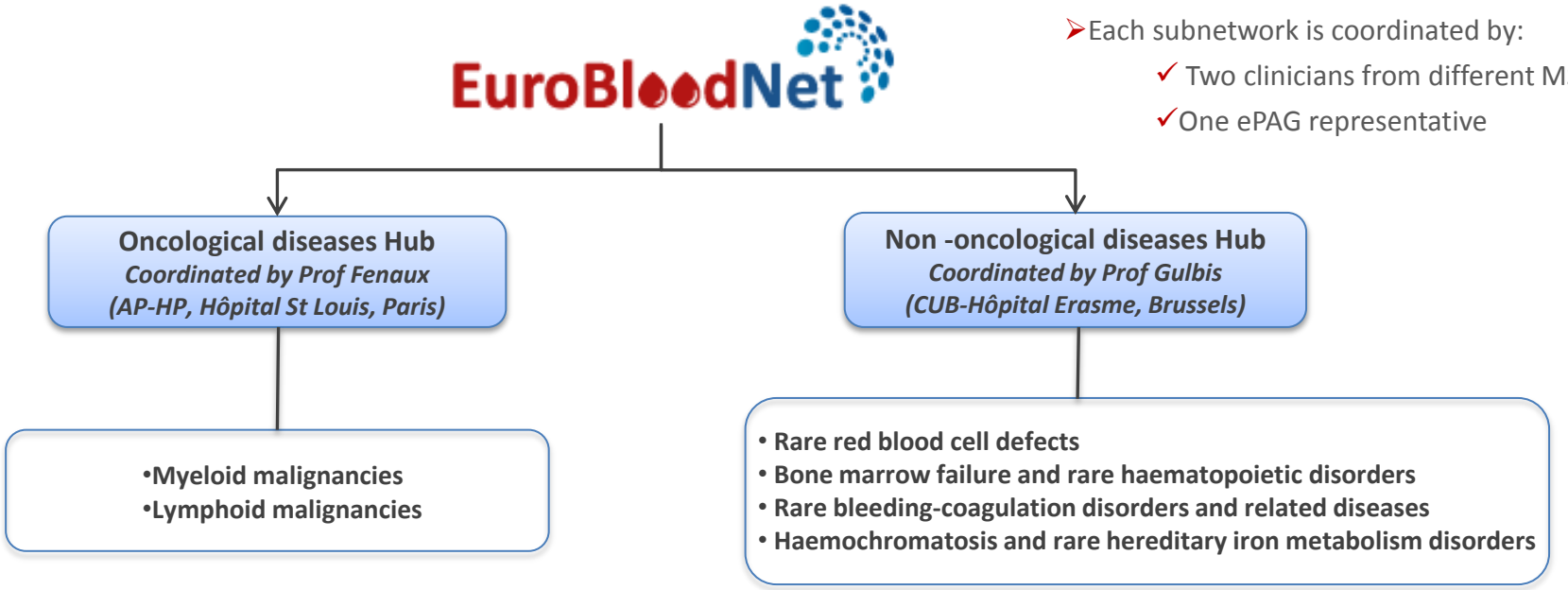
results from a joint effort of many pieces

ERN-EuroBloodNet is a collaborative network of **66 healthcare providers (HCPs) in 15 MS** that brings together individuals and institutions committed to improving healthcare services in **Rare Hematological Diseases**



Member Sate	nº HCP
Belgium	5
Bulgaria	2
Cyprus	1
Czech Republic	1
Germany	4
Spain	1
France	12
Ireland	1
Italy	21
Lithuania	1
The Netherlands	6
Poland	1
Portugal	3
Sweden	1
United Kingdom	6
Members	**66**

Coverage - Subnetworks



- Each subnetwork is coordinated by:
  - ✓ Two clinicians from different MS
  - ✓ One ePAG representative

Oncological diseases:

- •Myeloid malignancies - 23 HCPs from 9 MS

  P. Fenaux (France), U. Platzbecker (Germany) and S. Wintrich (UK)

- •Lymphoid malignancies - 22 HCPs from 10 MS

  A. Engert (Germany) , C. Thieblemont (France) and P. Aumont (France)

Non-Oncological diseases:

- •**Rare Red blood cell (RBC) defects** - 36 HCPs from 12 MS

  B. Gulbis (Belgium) , N. Cappellini (Italy) and L. Brunetta (Italy)

- •Bone marrow failure (BMF) and hematopoietic disorders - 20 HCPs from 8 MS

  A. Iolascon (Italy), R. Peffault (France) and M. Piggin (UK)

- •Rare bleeding-coagulation disorders and related diseases - 35 HCPs from 10 MS

  M. Makris (UK), F. Peyvandi (Italy) and A. Bok (UK)

- •Haemochromatosis and hereditary iron metabolism disorders - 15 HCPs from 6 MS

  G. Porto (Portugal) and D. Swinkels (The Netherlands)

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ERN-EuroBloodNet challenges

- To promote an equal access to high quality health care services for RHDs
- To disseminate cutting-edge knowledge and awareness on RHDs
- To facilitate EU MS policies on RHDs
- To improve EU mobility of expertise in the field of RHDs

→ **To reduce costs of RHDs**
to facilitate improvement in diagnosis and the delivery of high-quality, accessible and cost-effective healthcare for all patients with a medical condition requiring a particular concentration of expertise in medical domains where expertise is rare.

→ **To maximize the cost-effective use of resources by concentrating them where appropriate**

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Objectives and Transversal Fields of Action (TFAs)

ERN-EuroBloodNet objective is to promote **excellence for best health care** in rare hematological diseases based on cutting-edge diagnosis procedures and therapies while **removing barriers** for making them available at the European level

Objective 1: Improve **equal access to highly specialized healthcare** delivery for RHD across Europe.

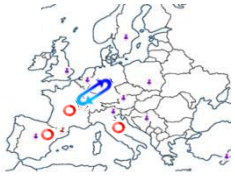
Objective 2: Promote **the best practices** in prevention, diagnosis and safe clinical care across Europe

Objective 3: Disseminate cutting-edge knowledge and facilitate **continuing medical education** in the field of RHD

Objective 4: Provide **inter-professional consultation** by sharing of expertise and safe exchange of clinical information

Objective 5: Foster **European cooperation** in highly specialized procedures for diagnosis, innovative treatments and research

Cross-border health



Best practices

Continuing medical education

Telemedicine

Clinical trials and research

Network for rare or low prevalence complex diseases

Network Hematological Diseases (ERN EuroBloodNet)

ERN-EuroBloodNet Cross Border Health challenges

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Objective

Improve equal access to highly specialized health care delivery for Rare Hematological Diseases (RHDs) across Europe

→ Establish a referral system for **patients and samples** in order to ensure the same level of access to healthcare across Europe

1) Mapping of Services (clinical and diagnosis) available in Europe for the best clinical care

2) Establishing a model for cross border referral system for patients and samples in accordance with Directive 2011/24/EU

3) Disseminating the policy report at the EU and national levels to foster policies and addressing specific needs

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Removing barriers

for making highly specialized services available to everyone

- Patient's rights and access to CBH services.
- Data protection and safe and security exchange of data.
- Rights and duties of health professionals involed in CBH services.
- Health services suitable for reimbursement at MS leved.
- Pan European framework for the exchange of human sample for diagnosis and research.

e.g. maximisation of cost-effective service:
Transfer of clinical records and samples: patient doesn't need to move from his country while carers could evaluate and assign diagnosis and treatment.

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ERN-EuroBloodNet Cross Border Health activities implemented

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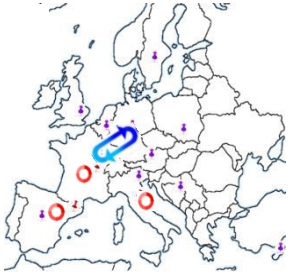
Cross border Health – activities implemented

Cross border health

Linked to Objective 1: Improve equal access to highly specialized healthcare delivery for RHD across Europe.

Coordinated by: J.Apperley (oncological hub), R. Colombatti (non-oncological hub) and A. Plate (ePAG representative)



- Mapping of services available in Europe
- Establishment of patients' pathways
- Establishment of a model for cross border referral system for patients and samples based on patients' pathways implementation

- **Deliverable 3- Report on EuroBloodNet members activity/facilities for RHD health provision (December 2017)**

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Exempli Gratia

ERN-EuroBloodNet Cross-Border Health Project

TFA: CROSS BORDER HEALTH CARE

Raffaella Colombatti, Coordinator for Non Malignant Hematology (Italy)
Jane Apperley, Coordinator for Malignant Hematology (UK)
Ananda Plate, Coordinator for ePAGs (UK)

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Bone Marrow Transplantation

Survey on BMT services for non malignant disorders will be uploaded on the EuroBloodNet website in the next few weeks.

The request to EBMT in order to collect data on BMT for Rare Hematological Disorders has been prepared and will be sent for the non malignant part in the next few weeks.

Data coming from the EBMT will be cross-checked with the ones of our questionnaire.

(*Prof. Raffaella Colombatti, HCP Azienda Ospedaliera di Padova, Italy*)
Pilot project of cross border health care for BMT in SCD among two HCPs of EuroBloodNet belonging to different countries
– challenges: Ethical, Administrative/Cost, Clinical Protocol

EuroBloodNet Questionnaire - TFA Cross Border Health Care
Non Malignant Disorders

THERAPEUTIC PROCEDURE: BONE MARROW TRANSPLANTATION

1. Do you consider Bone Marrow Transplantation (BMT) for your patients as a treatment option?

Yes, always☐ Yes, sometimes☐ No, never☐

2. For which Non Malignant Diseases do you consider BMT?

Sickle Cell Disease	☐	Inherited or acquired aplastic anemia	☐
Thalassemia	☐	Immune Deficiencies	☐
Metabolic Disorders	☐	Other	☐ _____

3. Does Your Center perform BMT?

Yes, but only for malignant diseases ☐ No☐ , because_____

Yes, for malignant and non malignant diseases ☐

Yes, Adults ☐

Yes Children ☐

4. For which Non Oncological Diseases does your center perform BMT?

Sickle Cell Disease	☐	Inherited or acquired aplastic anemia	☐
Thalassemia	☐	Immune Deficiencies	☐
Metabolic Disorders	☐	Other	☐ _____
None	☐		

5.If you consider BMT for your Non Oncological patients but DO NOT have possibility to transplant them at your Center, do you refer them to another Center?

Yes, in my Regional Area	☐	No, the patients have to go by themselves to another center	☐
Yes, In my country	☐	No, I continue with other treatments at my center	
Yes, abroad	☐		

6.In case of referral, do you have a standardized procedure?

Yes ☐ No ☐ Not yet, but working on it ☐

7.Does the referral to another center need your institution's approval?

Yes, only endorsement letter	☐	No	☐
Yes, financial	☐	Not from my Institution but other regulatory body	☐
Yes, medical	☐	I do not know, we never considered it	☐
Yes, Ethical	☐		

8.If you had to refer a patient, weather in your country or abroad, can you briefly describe the procedure/problems that you encountered?

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BMT

Hospital Saint-Louis, in Paris, has calculated an average cost of: **410 000,00 €**
for a *Hematopoietic stem cell transplantation (HSCT)*) for both patient and donor costs.
Post-Transplant costs are not included in this approximate calculation.

→ Reimbursement between MSs/ Reimbursement to patients
→ Prior authorization (one night hospital accomodation, highly qualified/ cost-intensive services)
→ MSs benefits baskets

CHAPTER III

REIMBURSEMENT OF COSTS OF CROSS-BORDER HEALTHCARE

Article 7

General principles for reimbursement of costs

1. Without prejudice to Regulation (EC) No 883/2004 and subject to the provisions of Articles 8 and 9, the Member State of affiliation shall ensure the costs incurred by an insured person who receives cross-border healthcare are reimbursed, if the healthcare in question is among the benefits to which the insured person is entitled in the Member State of affiliation.

- Health services suitable for reimbursement at the Member State level

Directive 2011/24/EU has no impact on the rights of each Member State to determine which health benefits they will provide. Thus, if a particular treatment is not reimbursed in a patient's home country, it will not be reimbursed if accessed in another Member State. Member States would be able to require prior authorization for "hospital care" and reimbursement would match the amount that patients would receive in their home country.

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EuroBloodNet Questionnaire on NGS – highly specialized diagnostic

Challenges raised by cross-border testing of rare diseases in the European union

Pia Pohjola*,[1], Victoria Hedley[2], Kate Bushby[2] and Helena Kääriäinen[3]

A study has already analysed the global situation on CBH NGS EU

Table 4 Reasons for offering and ordering cross-border testing and related problems

Reasons for offering cross-border testing	*Reasons for sending samples abroad*
Scientific interest	The test is not performed in one's country
Courtesy basis	More economical to test abroad
Profitable nature of testing	Faster turnaround time
Unique service	
Problems experienced	*Problems experienced*
Difficulties in receiving payments	Sending abroad not allowed in some countries
Variable procedures of invoicing	Difficulties in finding a reliable testing laboratory
Variable procedures of reimbursement	Difficulties in contacting the laboratory (uninformative webpage, language)
	Difficulties in sample logistics (customs, paperwork)
	Related bureaucracy
	Variable pricing of tests
	Variable procedures of reimbursement
	Variable procedures of invoicing
	Lack of specific funding for cross-border tests

European Journal of Human Genetics

ERN-EuroBloodNet Quick Slideshow on other implement activities for reaching the equal access to highly specialized care

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ERN-EuroBloodNet Telemedecine challenges

e.g: **Reduce the costs impact of RHDs**: high quality and cost-effective service: experts group virtually gathered together. Exchange of clinical records.

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Telemedicine

Linked to Objective 4: Provide inter-professional consultation by sharing of expertise and safe exchange of clinical information

Coordinated by: A. Engert (oncological hub) B. Gulbis (non-oncological hub) and S.Wintrich (ePAG represenetative)

✓ **To promote the use of the CPMS among the members of the network as the platform for inter professional consultation of complex cases, especially for those sub-thematic areas lacking of an already established telemedicine platform based on results from survey conducted.**

❖ **Activities implemented in the first year of ERN-EuroBloodNet**

1) **Participation in the pilot phase of the CPMS**

2) **Gathering of imput from subnetworks coordinators for the creation of an expert board**

3) **CPMS analysis in the context of the General Data Protection Regulation**

❖ **Results**

1) **5 Patients introduced in the CPMS during the pilot phase**

2) **Expert board constituted to be invited to join the CPMS during the 2nd year**

➢ **Deliverable 6 - Report on legal issues on inter-professional consultation of complex cases (February 2018)**

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ERN-EuroBloodNet Continuing medical education challenges

e.g. **Reduce the costs impact of RHDs** : High quality and specialized training courses focused on some RHD are very expensive and infrequent. ERN-EuroBloodNet will promote a blended educational courses , short stays and specialized training on-site and on-line .

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Continuing Medical education - activities implemented and results

Continuing medical education

Linked to Objective 3: Disseminate cutting-edge knowledge and facilitate continuous medical education in the field of RHD

Coordinated by: D. Bron (oncological hub), P. Aguilar-Martinez (non-oncological hub) and J. Geissler (ePAG representative)

- Elaboration of a multi annual educational programme and implementation of a blended educational program
- Co-organisation with the ePAGs of European symposia with interactive patient participation
- Identification of areas including highly specialized procedures requiring short stays for the acquisition of expertise

❖ Activities implemented in the first year of ERN-EuroBloodNet

1) **Collaborations with educational bodies:**

   1.1 European Hematology Association (EHA)

   1.2 European School of Haematology (ESH)

❖ Results

1) **Co-organization with EHA and the ePAGs of European symposia with interactive patient participation:** Collaboration of ERN-EuroBloodNet and ePAGs with EHA to reinforce the sessions dedicated to patients celebrated within the EHA congresses.

2) **Identification of different strategies for the methodology for gathering educational gaps to be implemented during 2nd year**

- **Deliverable 5- Report on actions foreseen by educational bodies (EHA and ESH) to address GAPS identified in annual educational programme (February 2018)**

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ERN-EuroBloodNet Clinical Trials and Research

e.g. Reduce the costs impact of RHDs: reinforce research, epidemiological surveillance like registries

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Clinical Trials and Research

Linked to Objective 5: Foster European cooperation in highly specialized procedures for diagnosis, innovative treatments and research

Coordinated by: M. della Porta (oncolgical hub), A. Piga (non-oncological hub) and A.L. Brunetta (ePAG representative)

- Promote the creation of a European registry of patients affected by a RHD
- Promote the participation in clinical trials
- Promote research

❖ Activities implemented in the first year of ERN-EuroBloodNet

1) Analysis of the state of the art of existing registries for RAs in collaboration with RADeep

1.1 Survey conducted through ERN-EuroBloodNet website

1.2 Desk research

➢ **Deliverable 7 - Report on existing registries for RHD (February 2018)**

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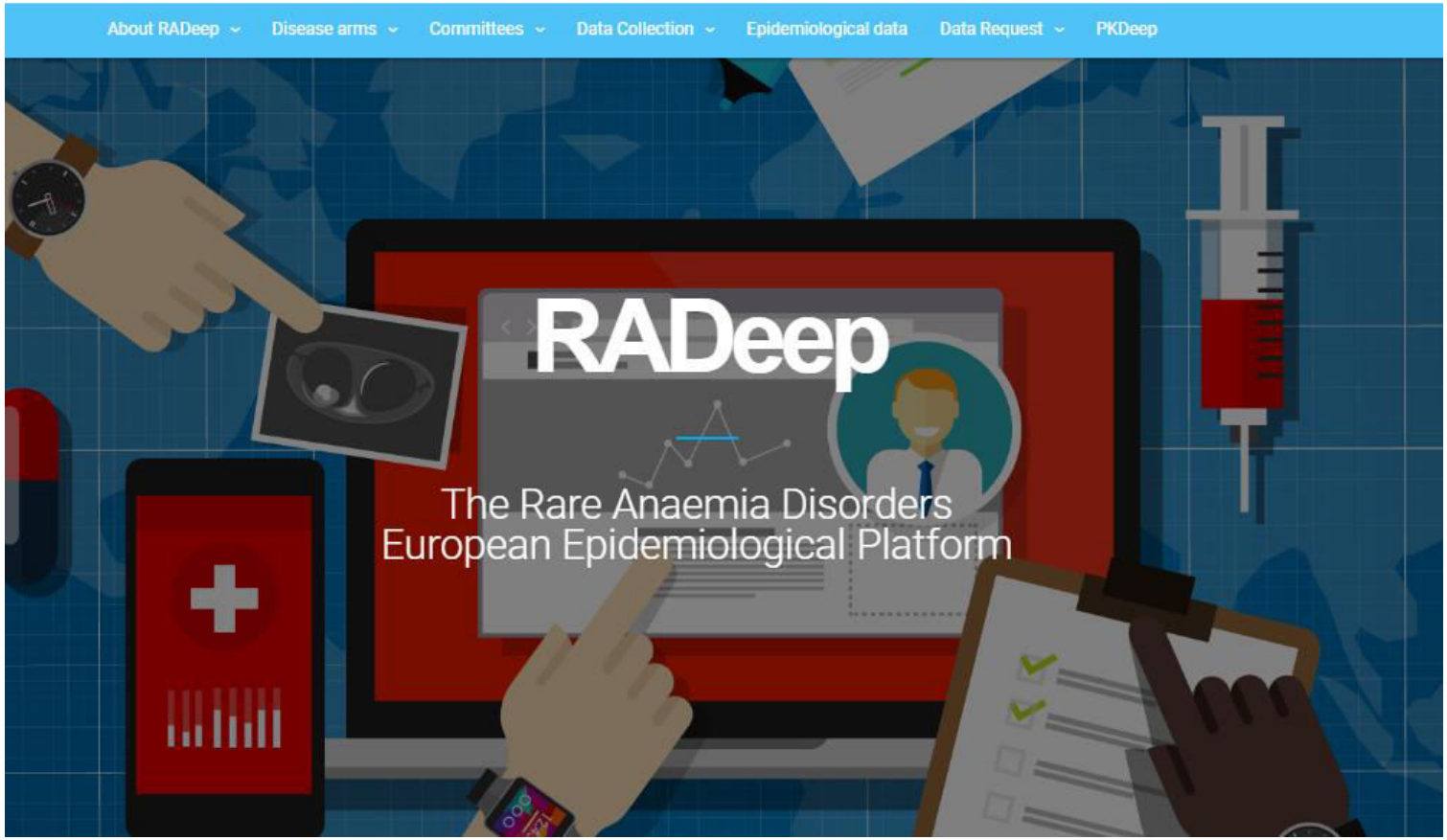
RADeep, the **Rare Anaemia Disorders European Epidemiological Platform**, is a joint venture conceived in the core of ERN-EuroBloodNet, as an **umbrella for both new and already existing European patients' registries in rare anaemias** (RAs).



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RADeep's Principle is to maximize public benefit from data on RAs opened-up through the platform with the only restriction needed to guarantee patient's rights and confidentially in agreement with EU regulations for cross-border sharing of clinical data.

RADeep general objective **is to map at the European level the demography, survival rate, diagnosis methods, main treatments and clinical features of RA patients** in order to facilitate the shaping of policies at both the national and the European level **to improve the delivery of best healthcare, including new therapeutic options.**

As in other RDs, **high quality epidemiological data based on an European approach is crucial for engage –OMICS based research and clinical trials by the identification of suitable patients groups** with common demographics and primary clinical disease manifestations. Basic clinical information is required to describe the clinical status of patients that can comply with the inclusion criteria for a clinical trial, while other patients can be excluded if a registry is well-designed.

RADeep also addresses **disease specific needs** through the promotion of best practices sharing i.e. facilitating access to **adequate diagnosis** methods through ERN-EuroBloodNet members, fostering the creation and implementation of **guidelines** for prevention and best clinical care.

SECURE SHARING AND RE-USE OF INFORMATION

EUROPEAN LEVEL INTEROPERABILITY

HIGH QUALITY DATA FOR RARE ANAEMIAS RESEARCH

MAXIMUM PUBLIC BENEFIT FROM DATA COLLECTED

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ERN-EuroBloodNet Best Practices

e.g. Reduce the costs impact of RHDs: share and spread information, knowledge and best practice and to foster developments of the diagnosis and treatment of rare diseases, within and outside the networks.

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Best practices

Linked to Objective 2: Promote the best practices in prevention, diagnosis and safe clinical care across Europe

Coordinated by: L. Malcovati (oncological hub), A. Iolascon (non-oncological hub) and A. Bok (ePAG representative)

- Create a comprehensive public database of reliable evidence based guidelines
- Foster the creation of new guidelines in collaboration with EHA and their transposition at the national level

- **Deliverable 4- Protocol for creation of the repository of reliable guidelines (December 2017)**

❖ Activities implemented in the first year of ERN-EuroBloodNet

1) **Definition of the protocol for the creation of the repository of reliable guidelines,** based in two complimentary approaches:

1.1 Creation of a list of international guidelines – Imput from subnetworks coordinators

✓ To compile the most frequent guidelines used for the main RHD conditions

1.2 Online questionnaire – Imput from ERN-EuroBloodNet members

✓ To complement the list of international guidelines for the coverage of very rare diseases or at national level

To reduce costs of RHDs

A multidisciplinary approach based on combined transversal field of actions to reduce the financial impact of RDs:

1) Establishing a referral system for patients and samples in order to ensure the same level of access to healthcare across Europe.

2) Analysing the legal framework for the establishment of CBH procedures

3) Pilot Projects to test CBH

4) high quality and cost-effective service: telemedicine and continuing medical education

5) Reinforce research, epidemiological surveillance and access to drugs

6) Promoting best practices

7) Accelerating some time-consuming procedures (diagnosis, delivery of best healthcare, administrative, etc.)

- To promote an equal access to high quality health care services for RHDs
- To disseminate cutting-edge knowledge and awareness on RHDs
- To facilitate EU MS policies on RHDs
- To improve EU mobility of expertise in the field of RHDs

→ **Maximizing the cost-effective use of resources by concentrating them where appropriate**

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Thank You!!!

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ERN-EuroBloodNet coordination team – Contact us!

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