



*The European Reference Network on
Rare Hematological Diseases*

ERNs: main challenges for the future. The experience of EuroBloodNet

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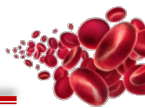
Co-funded by
the Health Programme
of the European Union



**European
Reference
Network**

for rare or low prevalence
complex diseases

 **Network**
Hematological
Diseases (ERN EuroBloodNet)



ERN-EuroBloodNet

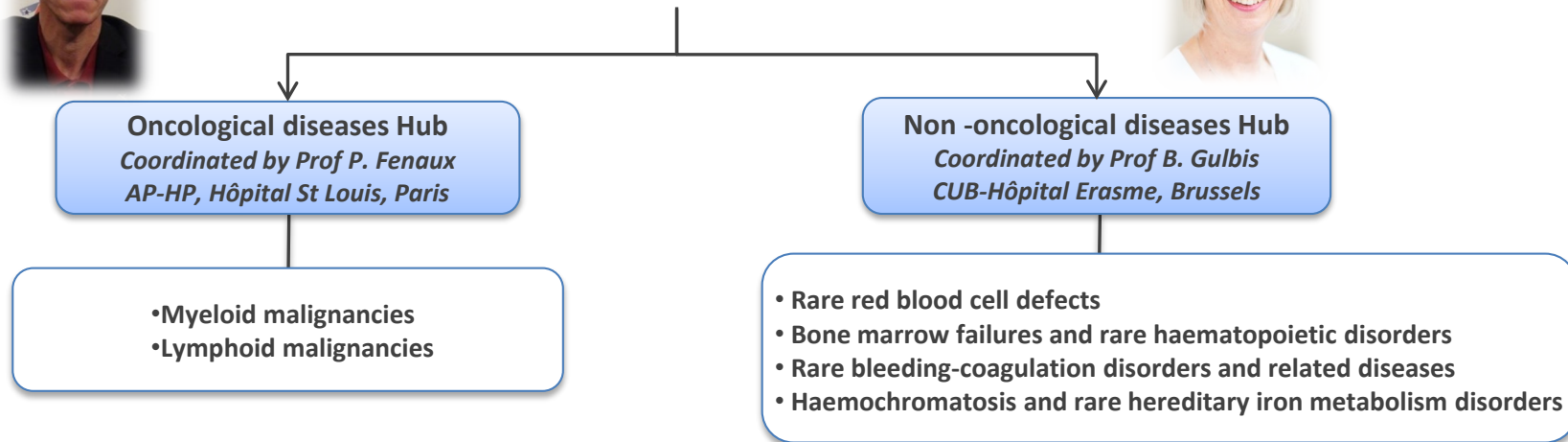
results from a joint effort of many pieces



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 State	n° 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



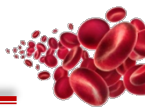
Oncological diseases coordinators:

- Myeloid malignancies
23 HCPs from 9 MS
P. Fenaux (France)
U. Platzbecker (Germany)
S. Wintrich (UK) - ePAG
- Lymphoid malignancies
22 HCPs from 10 MS
A. Engert (Germany)
C. Thieblemont (France)
P. Aumont (France) -ePAG

Non-Oncological diseases:

- Rare Red blood cell defects
36 HCPs from 12 MS
B. Gulbis (Belgium)
N. Cappellini (Italy)
L. Brunetta (Italy) - ePAG
- Bone marrow failure
20 HCPs from 8 MS
A. Iolascon (Italy)
R. Peffault (France)
M. Piggin (UK)
- Rare bleeding-coagulation disorders
35 HCPs from 10 MS
M. Makris (UK)
F. Peyvandi (Italy)
A. Bok (UK) - ePAG
- Hemochromatosis and hereditary iron disorders
15 HCPs from 6 MS
G. Porto (Portugal)
D. Swinkels (The Netherlands)





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ERN-EuroBloodNet

results from a joint effort of many pieces



- ❖ Promotes excellence in patient care, research, and education in hematology
- ❖ EHA's annual congress is the largest European event for hematology.
- ❖ Haematologica is the primary general hematology journal



- ❖ Pilot ERN in rare and congenital anaemias since 2002
- ❖ eHealth solutions: e-Registry, e-Learning, Telemedicine platforms
- ❖ WhiteBook : ENERCA recommendations for Centres of Expertise in Rare Anaemias



- ❖ Patient-driven alliance of organisations and individuals active in the field of RD in Europe
- ❖ Represents 724 RD organisations in 64 MS, covering more than 4000 RD
- ❖ ePAGs - European Patient Advocacy Groups, are the patients representatives for ERNs

And many more...



- ❖ Pilot ERN for the Haemophilia and other inherited bleeding disorders



- ❖ Network for patient care and research for inherited disorders of iron metabolism and heme synthesis



- ❖ Publicly funded research network of excellence for the cure of leukemia.



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**



Objective: Directive 2011/24/EU

Patient access to

- Correct diagnosis
- Highly specialized procedures
- Clinical trials - New therapies

Challenges:

- Lack of **awareness** on the procedure to follow for application on Crossborder Health directive
 - ❖ Are NCPs for cross border health really in place?
- **Reimbursement** process
 - ❖ payment in advance
 - ❖ WHAT is REIMBURSED in ONE COUNTRY MIGHT NOT BE THE SAME IN ANOTHER
 - ❖ Other costs not covered...the patient is not at home



PILOT PROJECT of cross border for BMT in Sickle Cell Disorders

IRELAND (Dublin) – ITALY (Padova)



June 2017: decision to work on this project based on Ireland's need and ongoing work in the field of SCD

June-December 2017: obtain "in theory" approval by both institutions with letter of Intent from both institutions

December 2017:

- beginning of draft of Clinical/Medical Protocol;

- beginning of Financial review of all previous BMT for SCD by Italian Hospital

June 2018: Ireland's Team visit to Padova; final review of Medical and Logistic Protocol

June-November 2018: review and draft of financial and administrative aspects

November 6 th 2018:

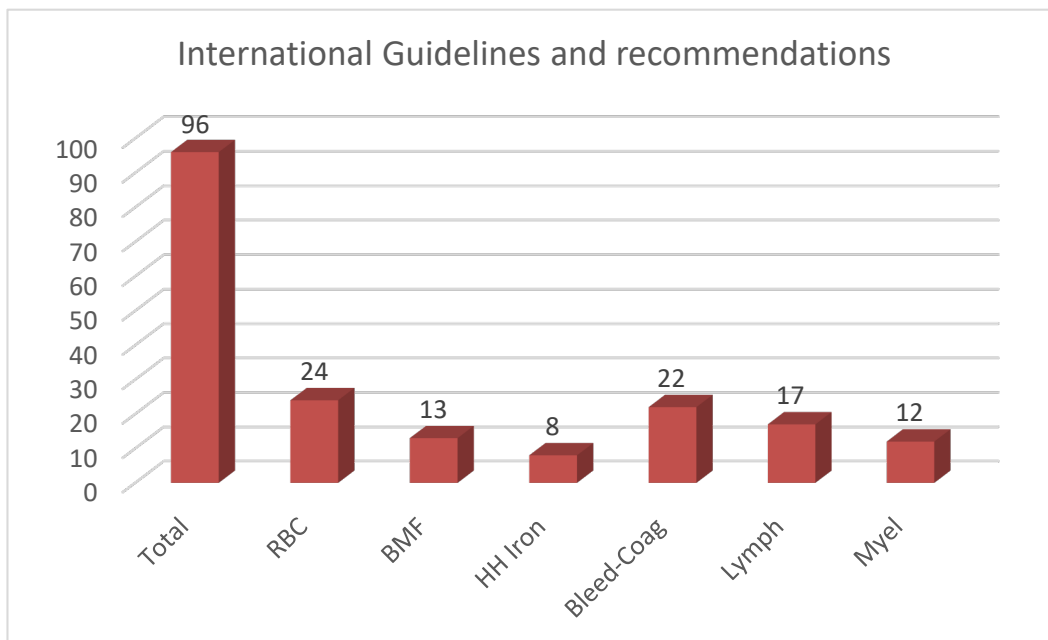
Approval by both institutions of the financial/administrative aspects

Agreement to be signed by the end of November



Create a comprehensive public database of reliable guidelines

Database: 96 international guidelines and recommendations



- **Level A: Evidence- and consensus-based guidelines / recommendations** involving assessment of the quality of scientific evidence (e.g. GRADE, SIGN) and formal consensus development techniques (e.g. Delphi method)

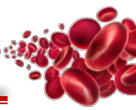
- **Level B: Consensus-based guidelines / recommendations** – Adopting formal consensus development techniques (e.g. Delphi method) without assessment of the quality of scientific evidence.

- **Level C: Expert opinion** only includes consensus among experts

Collaboration with UKNEQAS: Development of a new Scheme (Pyruvate Kinase Deficiency)

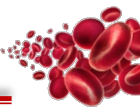
UK NEQAS
International Quality Expertise





Challenges:

- **Guidelines Lacking** for some groups of diseases – especially non malignant disorders
- Guidelines need to be reviewed and **updated**
- +++ are they really **implemented**?



Objective: Fill the existing gaps on the dissemination of cutting-edge knowledge in hematology in EU

Identification of educational GAPS in collaboration with EHA and ESH



A survey for the identification of educational gaps has been conducted:

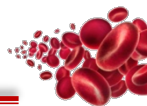
Challenges:

- A lot of education courses and material due to EHA and ESH in malignant hematology

- Gaps in education
 - for non-malignant hematology
 - For nurses, laboratory staff, patients
 - Material in different EU languages

- Importance of pharmaceutical companies in education



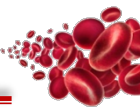


Short fellowships of health professionals in ERN-EuroBloodNet members with very specific expertise

Paroxysmal nocturnal hemoglobinuria (PNH) was identified during the first year of implementation as one of the areas that will benefit from organizing short stays.

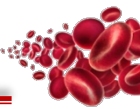
Sessions include:

- Interactive lectures on diagnosis, treatment and long term management
- Tutorials on clinical cases
- Attendance to ward round
- Attendance to diagnostic lab



Challenges for the Clinical Patient Management System (CPMS):

- **Reimbursement** of professionals/ Institutions at the HCP level ?
- How to avoid **simple cases**? - Filter for Rare Diseases
- **++ Cumbersome/ time consuming** procedure in daily practice for cases coming from centers outside of the ERN



Objective:

Registries
Clinical research

Mapping of Clinical Trials

Mapping of existing CTs with focusing on:

- *Diseases where there are no or very few clinical trials available*
- *Breakthrough new drugs available in only few member states*

Challenges:

- How to organize clinical research (sponsorship...) and how to fund it?
 - Official sponsor
 - funding



After almost 2 years of activity , ERNs are becoming active platforms for the promotion of best health care for RDs patients. **However...**

a) ERNs are not sufficiently integrated in **National healthcare systems**

Creation of **ERNs Boards of MS Contact Points** to lobby with

- National authorities
- Scientific societies

b) ERNs do not receive the **funding** needed to deliver on their function

While potential impact of ERNs on patient quality of life is high, it will not be achieved unless these two issues are appropriately addressed

.... *Finally, the challenge of diseases for which there is limited patient representation (eg: sickle cell disease)*



Thank You

ERN-EuroBloodNet coordination team – Contact us!



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