"Capacity Building with Patient Empowerment"

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(slide credit to EUPATI UK & EuroBloodNet)

PNH SUPPORT

Mission: Empowering patients for their key role in health-related research

EUPATI
European Patients' Academy
on Therapeutic Innovation

BfArM MHRA
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Competent authorities

Public

Policy makers
/Research Policy

EUPATI
European Patients' Academy
on Therapeutic Innovation

Research
Ethics Committees

HTA agencies
& committees

Clinical
Research

EUPATI addresses three audiences

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European Patients' Academy on Therapeutic Innovation

EUPATI Patient Experts Training Course
-- for expert patients

2 courses done
96 fellows

100 expert patients

- ENGLISH

EUPATI Educational Toolbox
-- for patient advocates

>1,000,000 users to date

12.000 patient advocates

10 languages

- ENGLISH
- FRENCH
- GERMAN
- SPANISH
- POLISH
- ITALIAN
- RUSSIAN

EUPATI Internet Library
-- for the health-interested public

different approach

100.000 individuals

Two EUPATI training courses: Mission accomplished

European Patients' Academy on Therapeutic Innovation

- 96 graduates (EUPATI fellows) completed the two courses
- 58 disease areas, 31 countries
- 3rd course started in September 2017

HOW WELL HAVE WE BEEN DOING?

From time to time we look at our performance to understand the impact of EUPATI. This latest survey assessed the change in roles of the EUPATI Fellows who graduated from the EUPATI Expert Training Course. The trends that somewhat fewer patient advocates work as volunteers and more as paid staff, while the number of patient experts advising pharmaceutical companies has more than doubled, speak of professionalisation. The increase in the number of speaking assignments suggests that the Fellows' expertise is increasingly acknowledged. Regulators and payers are also relying on advice from Fellows more than before. The number of inactive patient organisations members has dropped as EUPATI Fellows become more and more integrated into the

www.eupati.eu

What is the EUPATI Toolbox on Medicines R&D?

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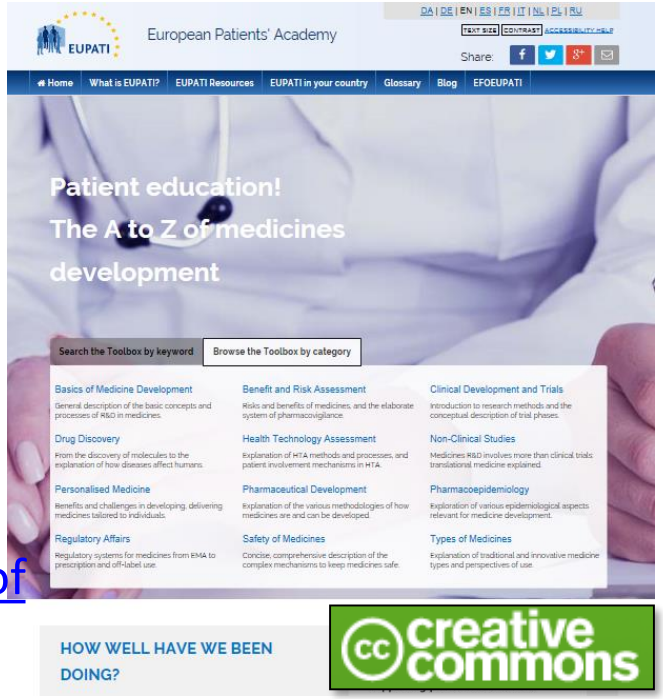
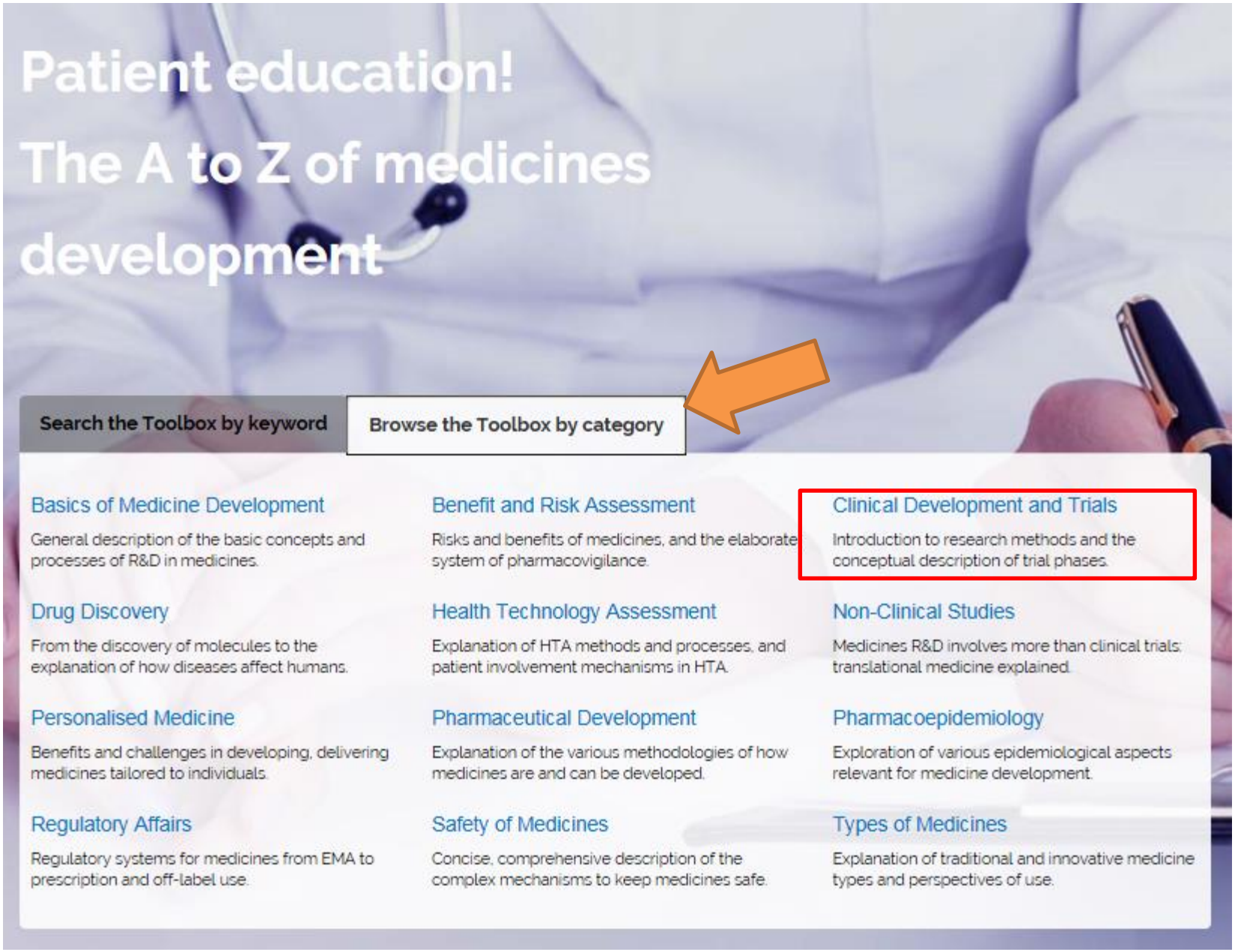
- elaborate web resource for lay people to inform themselves about all processes of medicines R&D
- provides texts, infographics, PPT slide decks and videos, all released under the 'Creative Commons License'.
- All content is available in 9 languages
- To ensure the content is accurate, accessible and objective, it has gone through a complex process of authoring, review and validation.
- The 'Starter Kits' support preparation of short courses for patients/patient advocates based on content from the Toolbox.




Patient education!
The A to Z of medicines development
Search the Toolbox by keyword
Browse the Toolbox by category
Basics of Medicine Development
General description of the basic concepts and processes of R&D in medicines.
Benefit and Risk Assessment
Risks and benefits of medicines, and the elaborate system of pharmacovigilance.
Clinical Development and Trials
Introduction to research methods and the conceptual description of trial phases.
Drug Discovery
From the discovery of molecules to the explanation of how diseases affect humans.
Health Technology Assessment
Explanation of HTA methods and processes, and patient involvement mechanisms in HTA.
Non-Clinical Studies
Medicines R&D involves more than clinical trials: translational medicine explained.
Personalised Medicine
Benefits and challenges in developing, delivering medicines tailored to individuals.
Pharmaceutical Development
Explanation of the various methodologies of how medicines are and can be developed.
Pharmacoepidemiology
Exploration of various epidemiological aspects relevant for medicine development.
Regulatory Affairs
Regulatory systems for medicines from EMA to prescription and off-label use.
Safety of Medicines
Concise, comprehensive description of the complex mechanisms to keep medicines safe.
Types of Medicines
Explanation of traditional and innovative medicine types and perspectives of use.

Nine ‘starter kits for mini-courses’ cover topics in the following areas

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European Patients' Academy on Therapeutic Innovation

Starter Kits

1. Setting research priorities
2. Ethics Committees
3. Data Monitoring Committees
4. Trial Steering Committees
5. Scientific advice
6. Protocol design
7. Product information, informed consent and patient information to trial participants
8. Medicines safety
9. Health Technology Assessment

R&D topics covered in these starter kits

- Basics of Medicine Development
- Benefit and Risk Assessment
- Clinical Development and Trials
- Drug Discovery
- Health Technology Assessment
- Non-Clinical Studies
- Personalised Medicine
- Pharmaceutical Development
- Pharmacoepidemiology
- Regulatory Affairs
- Safety of Medicines
- Types of Medicines

Search

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Search Results for: protocol

Mini-course starter kit – Protocol design ARTICLE

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The starter kits provide you with links to relevant background reading in the toolbox and associated PowerPoint slide decks and media in order to prepare a single or multi-day training on protocol design.

Protocol GLOSSARY TERM

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The protocol of a clinical trial is a document that contains: The objectives (aims) of the trial The trial design, including: How participants will be selected; How many participants are needed; What measurements and endpoints will be used; and How bias will be minimised How the safety of people taking part, and the privacy of Read more

Per Protocol Analysis GLOSSARY TERM

Print Save as PDF

An analysis that is restricted to the participants who fulfil the protocol in terms of the eligibility, interventions, and outcome assessment. This analysis restricts the comparison of the treatments

Search

Search

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Mini-course starter kit – Protocol design

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Index

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2 Protocol Design
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3 Terms of use - Creative Commons
3.1 Use of the EUPATI logo

Introduction

This EUPATI Mini-course starter kit is designed for patient involvement in protocol design.

EUPATI Mini-course starter kits have been derived from content found in the EUPATI toolbox and EUPATI Patient Expert Training Course. The starter kits are thought to address roles that patients play in medicines development for example those shown in the figure below.

The starter kits provide you with links to relevant background reading in the toolbox and associated PowerPoint slide decks and media in order to prepare a single or multi-day training on the subject. Each of the starter kits contains a selection of PPT slides which you may use to educate patients/advocates about the "basics" in that area, e.g. in a two-hour to one-day seminar.

The starter kits are based on existing content from the EUPATI Toolbox, plus additional links to add-on Toolbox material. None of the "starter kits" are "ready-made course" modules – they are a ready-to-reuse resource for an experienced trainer to prepare and execute a course. You will need to edit them and put them into context.

Before you begin please download and review the 'Manual for Trainers'.

- Presentation: Manual for Trainers

  Size: 722,143 bytes, Format: .pptx

  A manual for trainers describing how to use the EUPATI mini-course starter kits to create trainings on patient involvement.

Protocol Design

This starter kit provides background reading, slides, a video, and quizzes to create training for patients who intend to become involved in protocol design.

Core reading

Making a medicine. Step 1: Pre-discovery

Making a medicine. Step 2: Target selection

Making a medicine. Step 3 and 4: Choosing a molecule or lead

Starter kits provide resources to prepare and run mini-courses

- **The Trainers' Manual** provides introduction to the starter kits including how to use them to prepare mini-courses.
- **PowerPoint decks** may be used to prepare a mini-course.
  - Core set of PPT slides, outlining a specific area of R&D and how patients can get involved.
  - Additional links to EUPATI Toolbox resources, including links to Toolbox elements, example case studies and exercises.
- **Some guidance** on how to select EUPATI Toolbox material that is most relevant for a specific mini-course
  - e.g. how to find texts, slide sets, images and illustrations in the EUPATI Toolbox that you may want to use.

Public licence model guarantees ownership and re-use by the public

EUPATI
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- **What we bring in and what we produce is free for unlimited use by the public**

creative commons

You may:

- Share tools: copy & redistribute in
- any medium/format
- Adapt tools: remix, transform, & build upon the material

You must:

- Give appropriate credit
- Provide a link to the license
- Indicate modifications
- Distribute adapted content under creative commons license

You may not:

- Use the licensed material for commercial purposes

EUPATI National Platforms

European Patients' Academy on Therapeutic Innovation

- Es**tablished in:**
  - Austria, France, Germany, Ireland, Italy, Luxembourg, Malta, Poland, Spain, Switzerland, the UK, Denmark, Slovakia and Serbia
- **Coming soon to:**
  - Belgium, Romania, Portugal, Greece, Hungary and the Netherlands

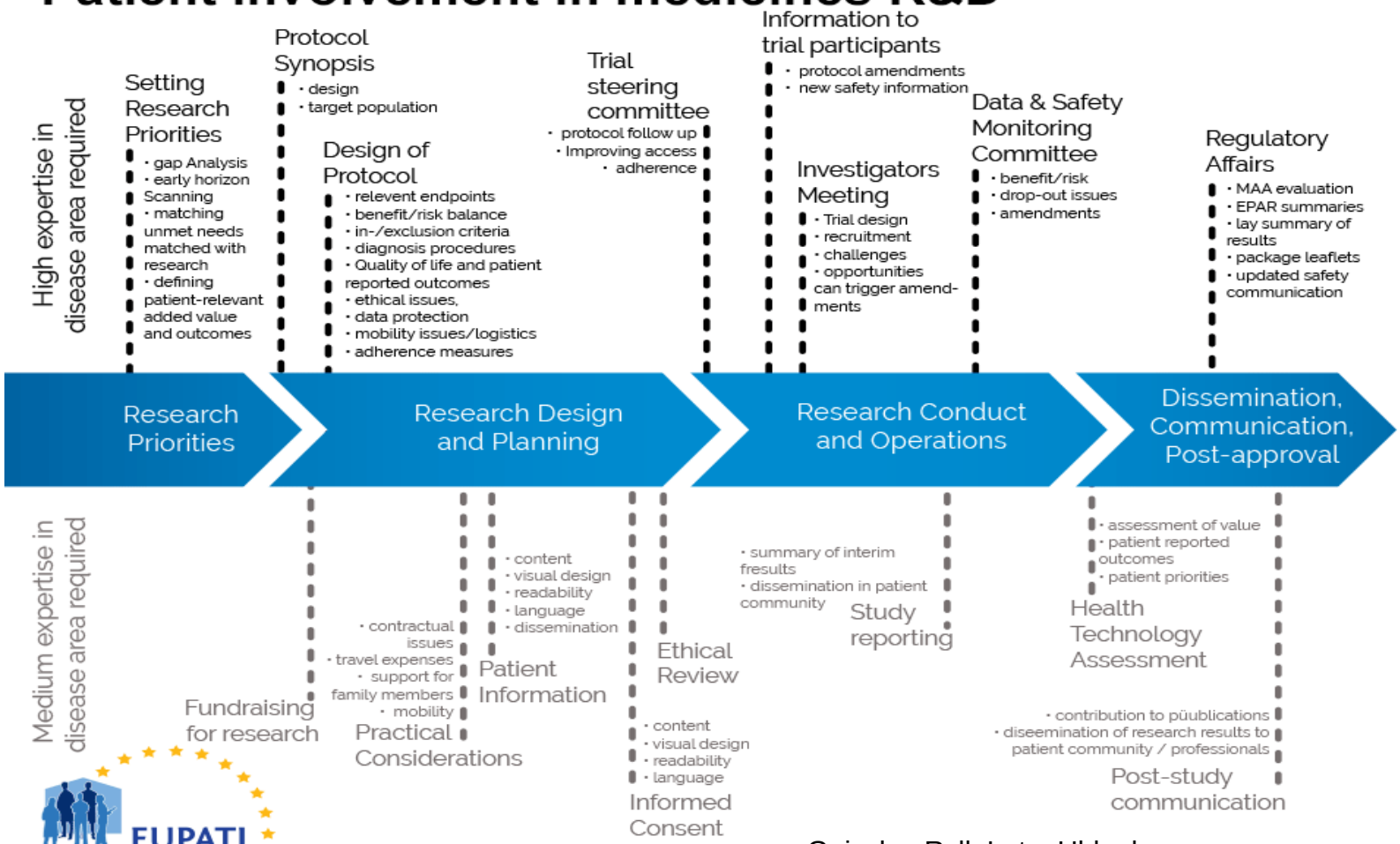
- **Purpose:**
  - bring patient, academic & industry partners together to discuss patient education & involvement in medicines R&D
  - raise awareness of the role of patients in medicines R&D

EUPATI Guidance documents on patient involvement

European Patients' Academy on Therapeutic Innovation

- EUPATI https://www.eupati.eu/guidance-patient-involvement/
  - Pharmaceutical industry-led medicines R&D
  - Ethics committees
  - Regulatory processes
  - Health technology assessments (HTA)

Patient involvement in medicines R&D



Geissler, Ryll, Leto, Uhlenhopp,
Therapeutic Innovation & Regulatory Science (2017),
doi: 10.1177/2168479017706405

Ensuring the Future of EUPATI Project

EUPATI
European Patients' Academy on Therapeutic Innovation

- Launched on 1 September 2018 for 24 months, until August 2020
- Co-led by: European Patients' Forum and Bayer
- Aims to ensure optimal exploitation & sustainability of core achievements of the IMI- EUPATI Project 2012 to 2017:
  - Patient Expert Training Course
  - Multilingual public toolbox
  - EUPATI National Platforms network

My experience as EUPATI Fellow

PNH SUPPORT

- Established PNH Support as a CIO in 2015
  - ➢ Engaged in R&D process with various pharma companies
    - Reviewing protocols, PIS, safety information, advising on design of qualitative research, design of QoL questionnaires, advising on unmet need
  - ➢ Consultee in NICE HST process
- Formed an umbrella European PNH Alliance
  - ➢ Participated in a haematology Community Advisory Board
- ePAG - European Reference Network, EuroBloodNet
- Member of the EHA Task Force on the Fair Pricing of Drugs
  - ➢ Speaker and co-chair at EHA Congress June 2018
- Co-chair of EUPATI UK platform
  - ➢ Delivery of mini-courses to other patient representatives

EuroBloodNet

The European Reference Network on Rare Hematological Diseases

European Commission

European Reference Network
for rare or low prevalence complex diseases

Network Hematological Diseases (ERN EuroBloodNet)

www.eurobloodnet.eu

EuroBloodNet

- The first **24 ERNs** covering 24 different medical specialities were officially approved by the EC in December 2016 and started their activity in March 2017, one of them being **ERN-EuroBloodNet** - for rare haematological diseases (both oncological and non-oncological rare)
- **European Reference Networks (ERNs) involve healthcare providers across Europe**. They aim to facilitate discussion on complex or rare diseases and conditions that require highly specialised treatment, concentrated knowledge and resources.
- **7 ePAGs representatives** have been currently appointed for ERN-EuroBloodNet, all of them involved in the Scientific and Strategic Board of the network ensuring that patient's voice is fully represented
- Enhances cross-border healthcare/access to required expertise for all patients
- Expertise travels rather than the patient

https://www.eurobloodnet.eu

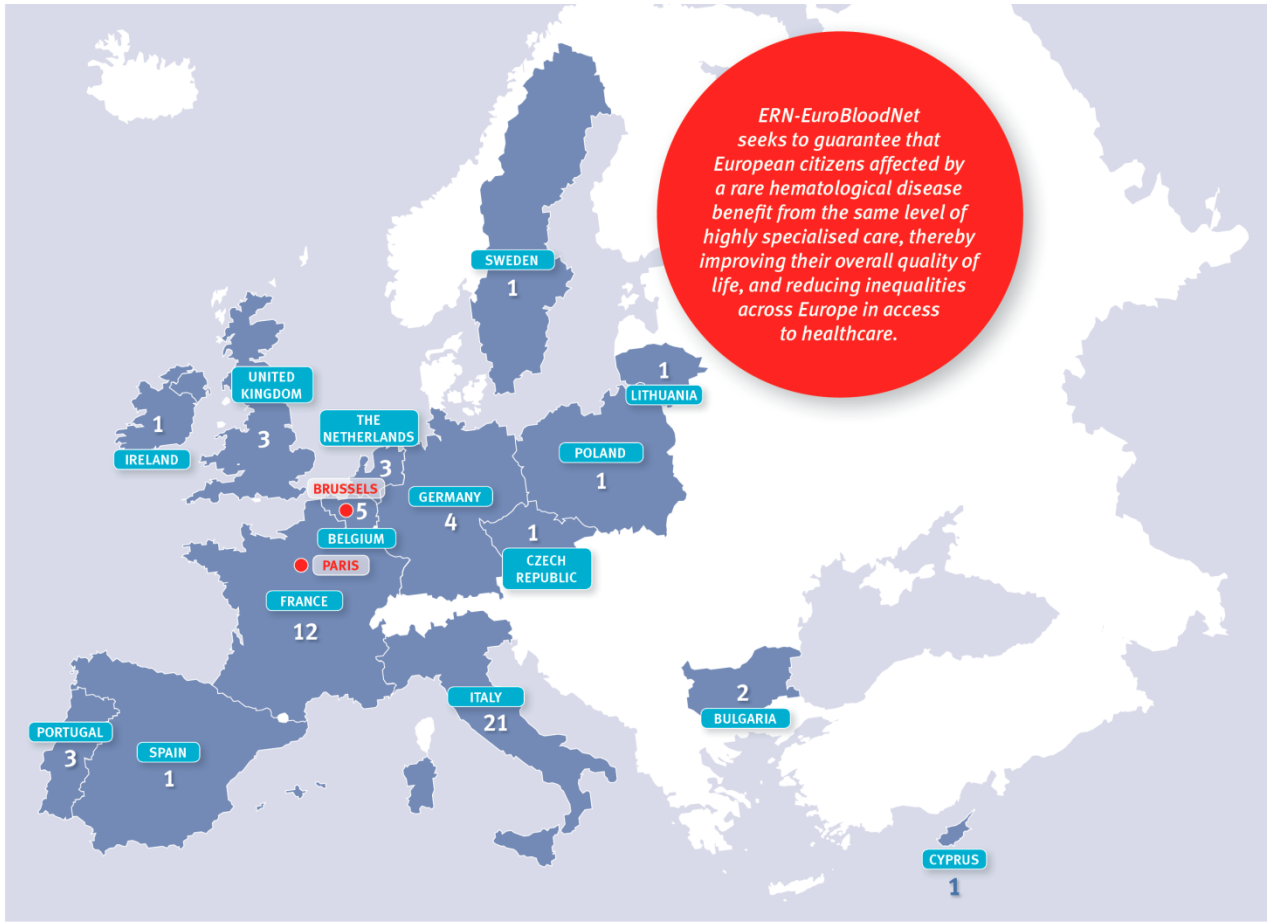
European Commission

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 State	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**

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

EC platforms for ERNs: Clinical Patients Management System and ERN Collaborative Platform

Clinical Patient Management System (CPMS)

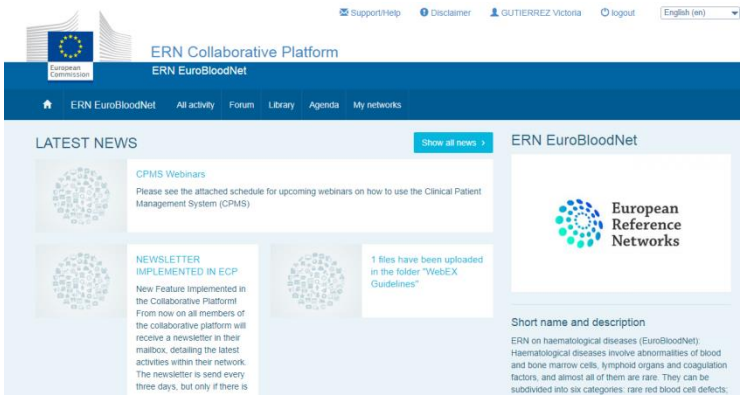
The focus is first on diagnosis and treatment for patients by providing tools for collaboration, **virtual consultations by sharing clinical data and medical images**. Steps:

1. Register the patient consultation and record the **patients' consent**
2. Share clinical, **pseudonymised data** on the patient Consultation process to arrive at clinical conclusions on diagnosis and treatment
3. Archive the patient case data, forming the ERN patient register

ERN Collaborative Platform (ECP)

Supports the ERN Board of Member States, the ERN Coordinators and ERNs members in their:

- Online communication
- Document management
- Event organisation.
- NOT to exchange clinical patient data



ECP is the platform for internal communication within the network.

Request your access to ERN-EuroBloodNet through:

https://webgate.ec.europa.eu/ern/