

# DELIVERABLE 5.2 ERN-EUROBLOODNET REPOSITORY OF CLINICAL PRACTICE GUIDELINES AND OTHER CLINICAL DECISION MAKING TOOLS ON RHD

ERN-EuroBloodNet: European Reference Network on Rare Hematological Diseases

EUROPEAN REFERENCE NETWORKS  
FOR RARE, LOW PREVALENCE AND COMPLEX DISEASES

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## DOCUMENT INFORMATION

### DELIVERABLE 5.2 ERN-EUROBLOODNET REPOSITORY OF CLINICAL PRACTICE GUIDELINES AND OTHER CLINICAL DECISION MAKING TOOLS ON RHD

Report document

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Authors:

**Maria del Mar Mañú Pereira – ERN-EuroBloodNet Scientific Director and representative in the “ERN Working Group on Knowledge Generation”**

**Luca Malcovati - ERN-EuroBloodNet representative in the “ERN Working Group on Knowledge Generation”**

**Victoria Gutiérrez Valle – ERN-EuroBloodNet Dissemination & IT Manager**

**Béatrice Gulbis - ERN-EuroBloodNet co-Coordinator and non-Oncological Hub chair**

**Pierre Fenaux - ERN-EuroBloodNet Coordinator and Oncological Hub chair**

#### Short Description

Report on the comprehensive public database of reliable guidelines based on results from desk research and survey conducted among members.

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## 1. INTRODUCTION

### CLINICAL PRACTICE GUIDELINES AND OTHER CLINICAL DECISION MAKING TOOLS FOR RARE HEMATOLOGICAL DISEASES, GAPS AND NEEDS

Clinical decision support systems represent nowadays essential tools for health professionals to guide on the next steps to take on the prevention, diagnosis, treatment and management of patients, considering the latest information and data available on the field and aiming to deliver value-based health care.

In the area of clinical decision support systems, different types of tools are identified based on the procedure followed for its development. As defined by the ERN Continuous Monitoring Working Group of the Member States and the ERN Coordinators, Clinical practice guidelines (CPGs) are defined as "statements that include recommendations intended to optimize patient care that are informed by a systematic review of evidence and an assessment of the benefits and harms of alternative care options". While on the other hand, other Clinical Decision Making Tools (CDMTs) "Clinical Consensus Statement is the end product developed by an independent panel of (at least 3) subject matter experts convened specifically to perform a systematic review of the available literature, for the purpose of understanding a clinically relevant issue or surgical procedure, offering specific recommendations on a topic. Compared to Clinical Practice Guidelines and Clinical Practice Recommendations, Clinical Consensus Statements undergo a less rigorous peer review process."

In this context, during past decades a great number of clinical decision support systems have been produced and implemented supporting the adequate provision of health care for millions of patients across the world. However, due to the low prevalence of Rare Diseases (RD), the development of high quality guidelines has usually been postponed for the prioritization of more prevalent diseases. Fortunately, the contribution of these support tools to shorten the time to diagnosis and improvement of the quality of care on RDs is now widely acknowledged, and several European countries have recently included their development as a priority in their national plans on RDs.

Another issue is the lack of findability of CPG and CDMT tackling RDs. According to "Clinical Practice Guidelines for Rare Diseases: The Orphanet Database", there are a large number of national and international databases gathering CPGs, but generally containing very few addressing RDs, and are difficult to find amongst the mass of recommendations available for more frequent diseases. Moreover, a significant number of the guidelines produced for RDs by research networks, reference centres or other organizations are not published in international peer-reviewed journals, and thus cannot be found in biomedical literature databases.

As for other RDs, this scenario remains when focusing on Rare hematological diseases (RHDs). A number of CPGs and CDMTs have been published on prevention, diagnosis, and clinical care of patients and many scientific societies have produced such guidelines at the national or European level. However there is no repository including quality assessment, evaluation of gaps or level of implementation, updates or any system to guarantee their usefulness and impact in patients' quality of life.

Accordingly, ERN-EuroBloodNet identified as a high priority to generate a comprehensive repository of CPG and CDMTs and the definition of a methodology for their classification based on quality domains and assessment of real translation into clinical practice with the final aim to, not only support guideline development when lacking, but also assess their implementation in the different EU Member States.

### ERN-EUROBLOODNET REPOSITORY OF CPGs AND OTHER CDMTs

#### List of international CPGs and other CDMTs

In the first two years of ERN-EuroBloodNet implementation (2017-2018), a protocol for the creation of the repository of CPGs and other CDMTs on RHD was defined aiming to achieve an accurate and reliable source of information on the guidelines and recommendations followed by the ERN-EuroBloodNet members. A first list of 69 CPGs and other CDMTs on RHD was identified with the feedback from subnetworks coordinators and members.

This first list was analyzed in terms of coverage of the different diseases encompassed by the network. Given the expertise of the subnetworks coordinators, the scope of the list was well balanced through the different RHDs, nevertheless, it was agreed to conduct a revision by additional experts identified in the field in order to produce a second version as extensive and comprehensive as possible. Special efforts were focused on the gathering of more CPGs for the oncological disorders given the relatively low number compiled in the first exercise. [Final list included a total of 116 CPGs and other CDMTs.](#)

#### Classification based on Quality domains

A methodology for classification of CPGs and other CDMTs based on Quality Domains was defined according to three domains: Scope and purpose, patients involvement and rigour of development.

As result for the classification exercise, a total of 68 CPGs and other CDMTs were classified with the participation of 10 ERN-EuroBloodNet experts as follows:

- 24 for Red blood cell disorders
- 13 for Bone marrow failures
- 6 for Haemochromatosis and other iron related disorders
- 26 for Myeloid diseases

The task is still ongoing for the Bleeding and coagulation disorders and Lymphoid malignancies.

## ERN WORKING GROUP ON KNOWLEDGE GENERATION AND EC TENDER

ERN-EuroBloodNet is one of the members of the “ERN Working Group on Knowledge Generation”, with the representation of Luca Malcovati, ERN-EuroBloodNet representative for best practices and chair of EHA Working group on guidelines, and Maria del Mar Mañú Pereira, ERN-EuroBloodNet scientific director. The working group has the common goal of producing unique guidelines at the EU level by the best experts in the field, following a concrete methodology and validated at the national level.

DG SANTE signed a contract for the development of CPGs and other CDMTs for rare diseases, entering into force in January 2020. The tender aims to develop *de novo* or appraise/adapt 2 new CPGs and 5 CDMTs per ERN according to the request and need of the ERNs. Tender includes the support with the provision of human and technical resources. In addition, a training program will be offered to educate 1 representative per network as expert in the field, and 5 representatives per network as intermediate experts.

## THE EUROPEAN HEMATOLOGY ASSOCIATION (EHA)

The [European Hematology Association](#) (EHA) is a non-profit association that promotes excellence in patient care, research and education in hematology by organizing an annual congress, providing an educational program based on the European Hematology Curriculum, publishing a journal, lobbying for hematology and promoting research. The association supports hematologists in and outside Europe to further improve hematology patient care;

EHA strongly supports good standards of care throughout Europe. Promoting the adoption of guidelines for diagnosis and treatments of hematologic diseases is an integral part of this effort, and EHA Scientific Working Groups (EHA-SWGs) represent the natural working engine of EHA for providing input and carrying out this task.

## 2. OBJECTIVES

One of the key objectives established by ERN-EuroBloodNet is to foster best practice sharing in RHD by creating a comprehensive public repository of reliable evidence based CPGs and other CDMTs, ranging from prevention, diagnostic tests and treatments to the organisation of patient-centred management in multidisciplinary teams.

The repository of guidelines and recommendations will serve as a central platform for sharing best practices, facilitating timely, effective and efficient translation of research results into patient oriented strategies at the individual and public health levels and provide professionals, patients, and policy makers with the best and most up to date information.

Based on the actions presented at the "Deliverable 5.1 ERN-EuroBloodNet repository of clinical practice guidelines and other clinical decision making tools on RHD" (February 2020), this Deliverable presents the actions undertaken to:

- a) Release of the central platform on CPGs and other CDMTs publicly at ERN-EuroBloodNet website including the search tool
- b) Identify the top 10 priority list for new CPGs and other CDMTs to be created/upgraded in line with the ERN WG on knowledge generation and EC tender
- c) Establishment of the collaboration with the European Hematology Association (EHA)

### 3. TASKS

## TASK 1. CREATION OF THE ERN-EUROBLOODNET PUBLIC REPOSITORY OF CPGs AND OTHER CDMTs AND SEARCH TOOL

Based on the list of CPGs and other CDMTs compiled and classified, a technical solution was implemented at ERN-EuroBloodNet website back office allowing the "tagging" of each of the document compiled and classified, including:

a) Subnetwork covered

b) Disease groups:

70 Rare Hematological Diseases - Disease groups (RHD-DG) were defined and implemented at the ERN-EuroBloodNet website back office in order to allow the classification of the contents of the web and expertise of the members' profiles (See Deliverable 4.1 ERN-EuroBloodNet repository of members, February 2020, to see in details the Disease Groups definition).

c) Quality Domains:

Domain 1: Scope and purpose: Each document shall be classified based on the objectives and clinical questions, on: Prevention, diagnosis, treatment.

Domain 2: Patients' involvement: The composition of the document development group shall be evaluated with reference to the involvement of patient advocacy organizations patients.

Domain 3: Rigour of development: The methodology adopted to develop the guideline / recommendation shall be assessed according to the following scale.

A: evidence and consensus-based guidelines / recommendations: grading system for strength of recommendation involving assessment of the quality of evidence (study design, study quality, consistency) (e.g. GRADE, SIGN)

B: consensus-based guidelines / recommendations: formal consensus development techniques (e.g. Delphi method, nominal group technique)

C: expert opinion: entirely based on opinions by experts without formal consensus development techniques

Furthermore, a search tool was developed for allowing the search of the CPGs and CDMTs by the dedicated tags assigned to the documents.

## TASK 2. IDENTIFICATION OF ERN-EUROBLOODNET TOP 10 PRIORITIES FOR CPGs AND OTHER CDMTs DEVELOPMENT

In order to identify the needs for each ERNs to for the development/appraise of CPGs and other CDMTs, a first round of consultation process was conducted by the contractors, aiming to identify diseases or conditions that most urgently require evidence-based CPGs/CDMTs to ensure the highest standards of quality care.

For the identification of the ERN-EuroBloodNet top 10 priorities for CPGs and other CDMTs, the following survey was circulated among ERN-EuroBloodNet members during September 2020 for the provision of the gaps for each disease/condition they considered there is a need for CPGs/CDSTs development/adaptation:

- 1) Name of the disease/condition:
- 2) Targeted patient population:
- 3) What particular aspects of the disease/condition are of interest to be addressed by your ERN? (select all that apply):
  - Epidemiology
  - Prevention
  - Diagnosis
  - Prognosis
  - Treatment
  - Patient, disease or condition management
  - Other\_Specify:
- 4) Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types



of ERN clinical decision support tools. Please, choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition (select all that apply):

- Clinical Practice Guideline
  - Clinical Consensus Statement
  - Evidence Report
  - Diagnostic-Monitoring-Therapeutic Pathway Evidence-based Protocol
  - Do's and Don'ts Factsheets
  - Quality Measure
  - Patient Information Booklet
- 5) If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

## TASK 3. COLLABORATION WITH THE EUROPEAN HEMATOLOGY ASSOCIATION

A collaboration with the European Hematology Association (EHA) Guidelines Committee has been established aiming to promote and support the creation and adoption of guidelines for diagnosis and treatment of hematologic diseases.

The goal of the collaboration is to avoid duplication of efforts by both entities, and promoting synergies to agree one set of recommendations, to avoid discrepancies and to optimize resources in producing such guidelines.

The following experts have been nominated to work actively on this frame, involving candidates who are ERN-EuroBloodNet members and members of the [EHA Working Group on Guidelines](#):

- [Luca Malcovati](#) - [Foundation IRCCS Polyclinic San Matteo, Pavia](#)
- [Paola Bianchi](#) - [Foundation IRCCS Ca'Granda Ospedale Maggiore Policlinico, Milan](#)
- [Noemi Roy](#) - [Oxford University Hospitals NHS Foundation Trust](#)
- [Carlo Dufour](#) - [IRCCS Istituto Giannina Gaslini, Genoa](#)

## 4. RESULTS

### LINKED TO TASK 1. CREATION OF THE ERN-EUROBLOODNET PUBLIC REPOSITORY OF CPGs AND OTHER CDMTs AND SEARCH TOOL

The repository 68 CPGs and other CDMTs classified based on Quality Domains was launched in November 2020, making open available for the RHD community at the dedicated repository section: <http://eurobloodnet.eu/best-practices/guidelines-repository/>

The screenshot displays the EuroBloodNet website's search interface. At the top, there is a navigation menu with options like Home, EuroBloodNet, Membership, Best practices (highlighted), Education, CPMS, ENROL, Patients' advocacy, COVID-19, and News. Below the menu, a search bar is present. The main content area is titled 'Search Guidelines and other CDMTs'. It includes an introductory paragraph explaining the repository's purpose and a note about ongoing classification for certain conditions. Below this, there is a search filter section with three dropdown menus: 'Disease group' (set to All), 'Scope and Purpose' (set to All), and 'Rigour of development' (set to All). A red 'SEARCH' button is located to the right of these filters. Underneath, the 'Guidelines by subnetworks' section features four colored buttons with right-pointing arrows: orange for 'Bone marrow failure', green for 'Haemochromatosis and other iron disorders', purple for 'Myeloid malignancies', and cyan for 'Red blood cell defects'. A 'SEE ALL GUIDELINES' button is positioned at the bottom right of the main content area.

In the main page, a direct button to "See all guidelines" is available, while direct boxes allow the selection of the list of documents by Subnetworks.

On the other hand, the search engine implemented allows in addition the specific search for by RHDs Disease Groups, Scope and Purpose, and Rigour of development, through an easy interface which allows the selection of the different tags for the filtered search:

## Search guidelines

**Disease group**  
Pyruvate Kinase Deficiency

**Scope and Purpose**  
All  
1. Prevention  
2. Diagnosis ✓  
3. Treatment  
4. Prevention and diagnosis  
5. Diagnosis and treatment  
6. Prevention, diagnosis, treatment

**Rigour of development**  
B - Consensus based

**SEARCH**

**Guidelines by subnetworks**

Bone marrow failure →

Myeloid malignancies →

Once the search is performed, each CPGs and other CDMTs have their dedicated "profiles" including the link to their access:

## Search guidelines

**Subnetwork**  
Red blood cell defects

**Disease group**  
Pyruvate Kinase Deficiency

**Scope and Purpose**  
2. Diagnosis

**Rigour of development**  
B - Consensus based

**SEARCH**

---

Recommended methods for the characterization of red cell pyruvate kinase variants. International Committee for Standardization in Haematology

**Subnetwork:** Red blood cell defects  
**Disease group/s:** Pyruvate Kinase Deficiency

**Scope and Purpose:** 2. Diagnosis  
**Patients' involvement?:** No  
**Rigour of development:** B - Consensus based  
**Link:** <https://www.ncbi.nlm.nih.gov/pubmed/41566>

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Addressing the diagnostic gaps in pyruvate kinase deficiency: Consensus recommendations on the diagnosis of pyruvate kinase deficiency

**Subnetwork:** Red blood cell defects  
**Disease group/s:** Pyruvate Kinase Deficiency

**Scope and Purpose:** 2. Diagnosis  
**Patients' involvement?:** No  
**Rigour of development:** B - Consensus based  
**Link:** <https://www.ncbi.nlm.nih.gov/pubmed/30358897>  
**Comment:**  
2019

A dedicated piece of news on the release of the repository and search tool was published at the website and disseminated through ERN-EuroBloodNet newsletter and social media channels (Twitter, Facebook and LinkedIn):

- [ERN-EuroBloodNet repository of Clinical Practice Guidelines and Other Clinical Decision Making Tools are searchable at the new website section!](#)

## LINKED TO TASK 2. IDENTIFICATION OF ERN-EUROBLOODNET TOP 10 PRIORITIES FOR CPGs AND OTHER CDMTs DEVELOPMENT

Results from the survey among members were compiled and analyzed at the end of September 2020, with a total of 30 answers. Answers were discussed among the ERN-EuroBloodNet task force on Guidelines including María del Mar Mañú, Luca Malcovati, Noemi Roy, Paola Bianchi and Carlo Dufour, with the agreement of the following Top 10 suggested areas to promote with the development or upgrade of CPGs and other CDMTs:

### Topic 1: Systematic mastocytosis

- Type of tool: Clinical Practice Guideline
- Particular aspects:
  - Diagnosis
  - Prognosis
  - Treatment
  - Patient, disease or condition management

### Topic 2: Chronic Neutropenia

- Type of tool: Clinical Practice Guideline
- Particular aspects:
  - Diagnosis
  - Treatment
  - Patient, disease or condition management

### Topic 3: Primary central nervous lymphoma (PCNSL)

- Type of tool: Clinical Practice Guideline/ Clinical Consensus Statement
- Particular aspects:
  - Diagnosis
  - Prognosis
  - Treatment

### Topic 4: Burkitt lymphoma (BL)

- Type of tool: Clinical Practice Guideline/ Clinical Consensus Statement
- Particular aspects:
  - Diagnosis
  - Prognosis
  - Treatment

### Topic 5: Blastic plasmacytoid dendritic cell neoplasm (BPDCN)

- Type of tool: Clinical Practice Guideline/ Clinical Consensus Statement/ Evidence Report
- Particular aspects:
  - Diagnosis
  - Prognosis
  - Treatment
  - Patient, disease or condition management

### Topic 6: Large granular lymphocytes leukemia

- Type of tool: Clinical Consensus Statement
- Particular aspects:
  - Diagnosis
  - Treatment
  - Patient, disease or condition management

### Topic 7: Long term complications including liver Disease & Osteoporosis in Haemoglobinopathies

- Type of tool: Clinical Practice Guideline
- Particular aspects:
  - Prevention
  - Treatment

#### Topic 8: Hemochromatosis

- Type of tool: Clinical Consensus Statement/ Do's and Don'ts Factsheets/ Patient Information Booklet
- Particular aspects:
  - Epidemiology
  - Prevention
  - Diagnosis

#### Topic 9: Congenital dyserythropoietic anaemias (CDAs)

- Type of tool: Clinical Consensus Statement/ Diagnostic-Monitoring-Therapeutic Pathway Evidence-based Protocol
- Particular aspects:
  - Epidemiology
  - Diagnosis
  - Prognosis
  - Treatment
  - Patient, disease or condition management

#### Topic 10: Functional platelet disorders

- Type of tool: Clinical Consensus Statement
- Particular aspects:
  - Diagnosis
  - Patient, disease or condition management

Top 10 priorities were reported through the online Survey conducted by the EC, see Annex I EuroBloodNet Top 10 CPGs/CDSTs priorities survey for full details.

## LINKED TO TASK 3. COLLABORATION WITH THE EUROPEAN HEMATOLOGY ASSOCIATION

ERN-EuroBloodNet and the EHA has signed a Memorandum of Understanding (MOU) containing the terms of collaboration, including an overview of the tasks and responsibilities of each party and entering into force January 2021.

In this context, the first expected joint project is the development of a clinical decision making tool on Methemoglobinemia, under the co-charing of Prof. Achille Iolascon and Dr. Paola Bianchi.



## 5. CONCLUSIONS AND NEXT STEPS

The [repository of CPGs and other CDMTs on RHD](#) is publicly available on ERN-EuroBloodNet website allowing both non-experts and experts in RHD to benefit from an exhaustive database with reliable and updated clinical decision support tools, promoting the delivery of highly specialised procedures and treatments and the harmonisation of care delivery across EU.

In addition, the [search engine](#) implemented allows the findability of the CPGs and other CDMTs classified on quality domains, promoting the searchability by concrete needs of the RHD community, including: subnetwork, RHDs Disease Groups, Scope and Purpose (Prevention, Diagnosis, Treatment, and Rigour of development), or Rigour of Development (A Evidence and consensus based, B Consensus based, C Expert opinion).

The Top 10 priority list for CPGs and other CDMTs development identified by ERN-EuroBloodNet has allowed the targeting of the most important areas to be tackled in the next steps on the EC tender for the development of 2 new CPGs and 5 new CDMTs.

In this context, the EC CPGs Working Group has developed and circulated among ERNs the procedures (Handbooks) to prioritize proposals specifically for CGPs, which includes as key next steps:

- The identification of a prioritization ERN Expert Panel
- The prioritization of the 2 topics for CPGs based on a concrete prioritisation criteria, which comprise four domains (Social Burden, Stakeholders' Interest, Healthcare provision and Public Health) and fourteen items.

Currently ERN-EuroBloodNet is establishing the prioritization Expert Panel, while in parallel is developing a template to facilitate to Expert Panel members the prioritization process based on the circulated criteria.

Moreover, the official collaboration with the European Hematology Association (EHA) allows the implementation of a common strategy among both bodies, including the development of a clinical decision making tool on Methemoglobinemia as first target.

# ANNEX I

## ERN-EUROBLOODNET TOP 10 CPGs/CDSTs PRIORITIES SURVEY





## ERN Guidelines project: Clinical Practice Guidelines/Clinical Decision Support Tools Needs survey

Fields marked with \* are mandatory.

### Contact information

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**Please, answer the following questions about the needs for Clinical Decision Support Tools in your Network.**

**\* Name of ERN:**

- |   |  |
|---|--|
| <input type="radio"/> ERN BOND                    | <input type="radio"/> ERN EURO-NMD         |
| <input type="radio"/> ERN CRANIO                  | <input type="radio"/> ERN EYE              |
| <input type="radio"/> Endo-ERN                    | <input type="radio"/> ERN GENTURIS         |
| <input type="radio"/> ERN EpiCARE                 | <input type="radio"/> ERN GUARD-HEART      |
| <input type="radio"/> ERKNet                      | <input type="radio"/> ERN ITHACA           |
| <input type="radio"/> ERN-RND                     | <input type="radio"/> MetabERN             |
| <input type="radio"/> ERNICA                      | <input type="radio"/> ERN PaedCan          |
| <input type="radio"/> ERN LUNG                    | <input type="radio"/> ERN RARE-LIVER       |
| <input type="radio"/> ERN Skin                    | <input type="radio"/> ERN ReCONNET         |
| <input type="radio"/> ERN EURACAN                 | <input type="radio"/> ERN RITA             |
| <input checked="" type="radio"/> ERN EuroBloodNet | <input type="radio"/> ERN TRANSPLANT-CHILD |
| <input type="radio"/> ERN eUROGEN                 | <input type="radio"/> VASCERN              |

**\* Name of respondent (first, last):**

María del Mar Mañú Pereira

**\* Email address of respondent:**

mar.manu@vhir.org

### Diseases/conditions that need to be analyzed:

---



\* **How many diseases/conditions** within the scope of your Network **do you think that require CPGs /CDSTs most urgently** according to clinical need and scientific uncertainty? (in the following pages we will ask you some questions about each one)

- 1    6  
 2    7  
 3    8  
 4    9  
 5    10

*Although we are aware that there must be more than 10 diseases/conditions within your ERN that need some type of CPG/CDST, we request up to a maximum of 10 because the ERN Guidelines project includes the development of a maximum of 2 CPGs/CDSTs and the appraisal/adaptation of a maximum of 5 CPGs /CDSTs for each ERN, therefore, in no case is it expected to be able to provide CPGs/CDSTs to more than 10 diseases/conditions per ERN.*

## Information on the **first disease/condition** candidate for CPG/CDST.

---

\* **Name** of the disease/condition:

Systematic mastocytosis

\* Targeted patient **population**:

indolent mastocytosis, smoldering mastocytosis, aggressive mastocytosis, mastocytosis with an associated hematologic neoplasm, mast cell leukemia

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology  
 Prevention  
 Diagnosis  
 Prognosis  
 Treatment  
 Patient, disease or condition management  
 Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline  
 Clinical Consensus Statement  
 Evidence Report  
 Diagnostic-Monitoring-Therapeutic Pathway

- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

None

## Information on the **second disease/condition** candidate for CPG/CDST.

\* **Name** of the disease/condition:

Chronic neutropenia

\* Targeted patient **population**:

patients aged 0- to adult age with chronic neutropenia including genetically determined, autoimmune, secondary to immune-dysregulation, Idiopathic forms typical of the adult/elderly patients

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis
- Prognosis
- Treatment
- Patient, disease or condition management
- Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

None

## Information on the **third disease/condition** candidate for CPG/CDST.

---

\* **Name** of the disease/condition:

Primary central nervous lymphoma (PCNSL)

\* Targeted patient **population**:

adults

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis
- Prognosis
- Treatment
- Patient, disease or condition management
- Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical Consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

Adapting existing ESMO guidelines would be a good starting point - <https://www.sciencedirect.com/science/article/pii/S0923753419316400?via%3Dihub>

## Information on the **fourth disease/condition** candidate for CPG/CDST.

---

\* **Name** of the disease/condition:

Burkitt lymphoma (BL)

\* Targeted patient **population**:

adults

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis
- Prognosis
- Treatment
- Patient, disease or condition management
- Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical Consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

None

## Information on the **fifth disease/condition** candidate for CPG/CD.

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\* **Name** of the disease/condition:

Blastic plasmacytoid dendritic cell neoplasm (BPDCN)

\* Targeted patient **population**:

BPDCN at any disease stage

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis
- Prognosis
- Treatment
- Patient, disease or condition management
- Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical Consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

None

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## Information on the **sixth disease/condition** candidate for CPG/CDST.

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\* **Name** of the disease/condition:

Large granular lymphocytes leukemia

\* Targeted patient **population**:

any patient with Large granular lymphocytes leukemia

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis
- Prognosis
- Treatment
- Patient, disease or condition management
- Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical Consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

None

## Information on the **seventh disease/condition** candidate for CPG/CDST.

\* **Name** of the disease/condition:

Long term complications including liver Disease & Osteoporosis in Haemoglobinopathies

\* Targeted patient **population**:

adults with SCD or Thalassemia with long-term complications

\*

What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis
- Prognosis
- Treatment
- Patient, disease or condition management
- Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical Consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

None

## Information on the **eighth disease/condition** candidate for CPG/CDST.

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\* **Name** of the disease/condition:

Hemochromatosis

\* Targeted patient **population**:

adults

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis

- Prognosis
- Treatment
- Patient, disease or condition management
- Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical Consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

It will be soon submitted for publication a position paper by the International Bioiron Society (involving all experts in the field) with an update of the Hemochromatosis nomenclature

## Information on the **ninth disease/condition** candidate for CPG/CDST.

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\* **Name** of the disease/condition:

Congenital dyserythropoietic anaemias (CDAs)

\* Targeted patient **population**:

Adult and paediatric patients

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis
- Prognosis
- Treatment
- Patient, disease or condition management
- Other



\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical Consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

None

## Information on the **tenth disease/condition** candidate for CPG/CDST.

---

\* **Name** of the disease/condition:

Functional platelet disorders

\* Targeted patient **population**:

Adult and paediatric patients

\* What particular **aspects of the disease/condition** are of interest to be addressed by your ERN? (select all that apply):

- Epidemiology
- Prevention
- Diagnosis
- Prognosis
- Treatment
- Patient, disease or condition management
- Other

\* Depending on the identified need of each ERN and according to the available scientific evidence, it is possible to develop different types of clinical decision support tools. Please, [click here](#) for definitions and information on the different types of ERN clinical decision support tools. Please, **choose the type of clinical decision support tool that best meets the expectations of the network for the proposed disease/condition** (select all that apply):

- Clinical Practice Guideline
- Clinical Consensus Statement
- Evidence Report
- Diagnostic-Monitoring-Therapeutic Pathway
- Evidence-based Protocol
- Do's and Don'ts Factsheets
- Quality Measure
- Patient Information Booklet

If there is already a high-quality tool that meets the identified needs, instead of developing a new tool, the existing one could be adapted. If there are any previous CPGs/CDSTs suitable to cover the need identified, please specify as much as possible (title, author, year, website link if available):

None

## Comments:

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**We welcome any additional information** about the selected diseases/conditions and/or required CPGs /CDSTs:

The order do not reflect any prioritization criteria but thematic areas

\* **Would you like to participate in a virtual interview** and talk further about your ERN CPGs/CDSTs needs (duration: 20-30 min)?

Yes  No

If so, a person from the ERN Guidelines Team will contact you through the email you have already provided us within the next months. Thank you!

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**The survey has been finished.**  
**Thank you very much for your cooperation.**

## Contact

[Contact Form](#)





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