ERN-EUROBLOODNET

EUROPEAN REFERENCE NETWORK ON RARE HEMATOLOGICAL DISEASES

2. CLINICAL CARE

2.3.1 (Extended) The ERN promotes the use of technologies such as telemedicine, e-Health records, remote consultation, health information portals, electronic transfer of prescriptions, and multidisciplinary e-Meetings designed according to the needs and requirements of patients and families.

Supporting Document 19. Description of activities carried out to promote the use of ICT clinical tools

ERN-EUROBLOODNET PROMOTION OF ICT TOOLS

ERN-EuroBloodNet has promoted the use of ICT clinical tools by:

1. Promoting the sharing of expertise and safe exchange of clinical information through the Clinical Patient Management System
2. Promoting interoperability of existing e-Health records and epidemiological surveillance by the development of two Eropean registries in the core of ERN-EuroBloodNet: The European Rare Blood Disorders Platform (ENROL) and the ERN-EurBloodNet Collaborative Platform on Red Blood Cell and COVID-19 patients (See also Supporting Document 33. Registries and databases that the ERN has developed).

CLINICAL PATIENTS MANAGEMENT SYSTEM

The objective of the Clinical Patient Management System (CPMS) is to support European Reference Networks (ERNs) in the diagnosis and treatment of rare disease or low prevalence complex disease or conditions across national borders.

ERN-EuroBloodNet has ensured the efficient use and wider outreach among the rare hematological diseases (RHD) health professionals through the promotion of the CPMS within and outside network members by:

- **Implementing the ERN-EuroBloodNet CPMS Helpdesk**: Providing key information on the CPMS and training materials (short guides and videos) to promote and facilitate the use of the platform, available through the different subsections: About CPMS, How to use CPMS, Find experts for panels
- **Dedicated sessions at ERN-EuroBloodNet meetings**: including Board of the Network and Progress Meetings to provide an overview of the platform and increase awareness among ERN-EuroBloodNet's community
- **Creating CPMS Virtual Boards:** CPMS Virtual Boards of international experts representing very rare disorders, registered in CPMS and meeting for online regular cross border consultation. This initiative allows to cover geographical gaps, providing the tool for medical consultation between experts and health professionals from the countries where expertise on particular very rare disorders is missing.

ANALYSIS OF THE NUMBER OF USERS AND PANELS OVER THE LAST FOUR YEARS

As a result 42 panels have been created for RHDs since the launch of the CPMS, from which 21 are closed with outcome report produced. Moreover, 80 active users were available at the platform for providing advice by the end 2021 at the platform.

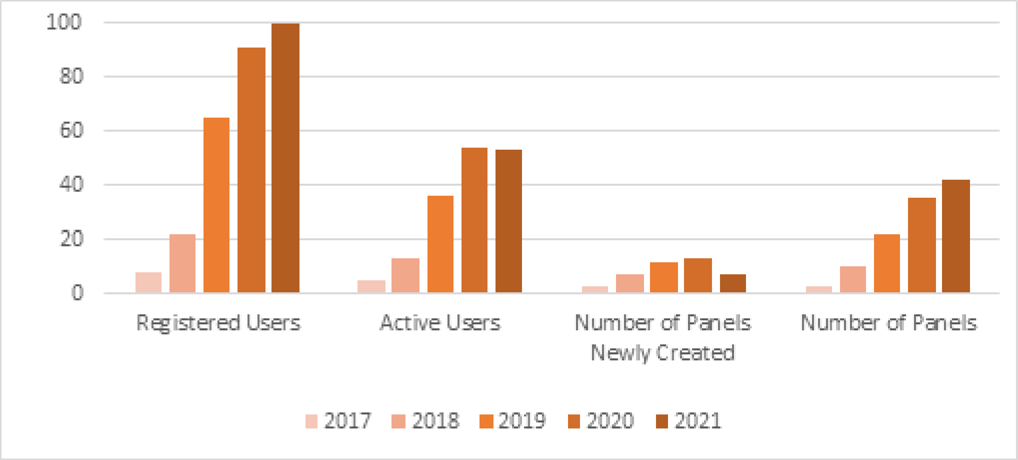

Fig 1. Yearly CPMS activity for ERN-EuroBloodNet until December 2021.

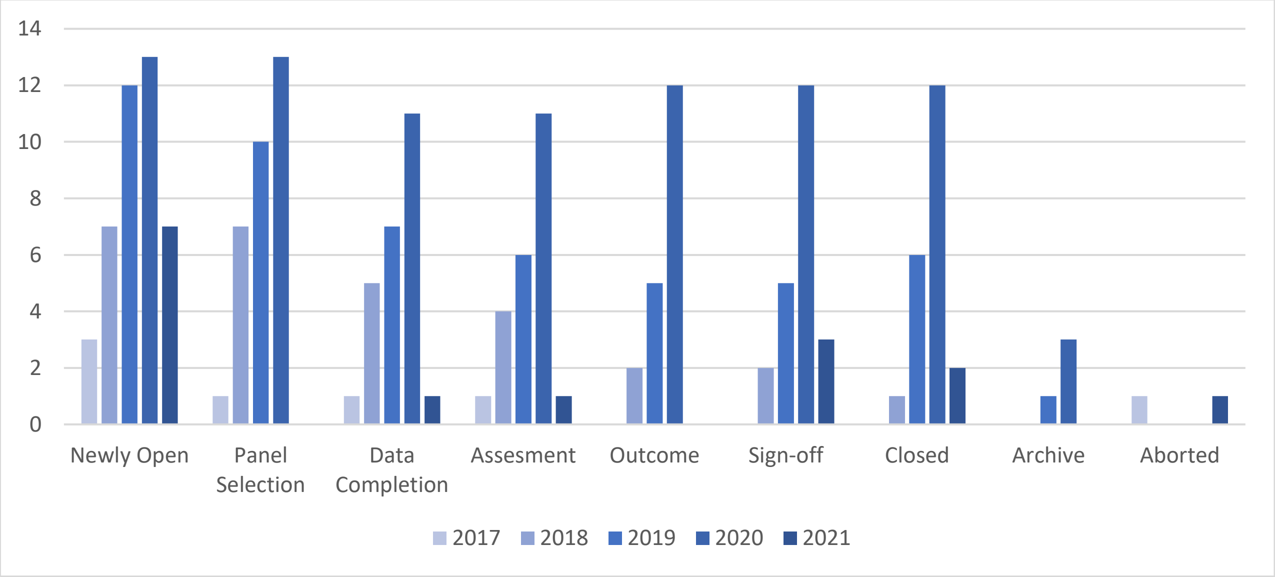

Fig 2. Yearly report on ERN-EuroBloodNet's CPMS Panel Stage.

INCREASE IN COUNTRY COVERAGE

The following map summarizes the number of registered users per country, accounting only those with an active account in SAAS authorization system (tool for the management of authorization requests from CPMS users). The map highlights some remaining gaps in the disease coverage especially in the central-Balkan Europe, which engagement will be promoted in the next period of implementation (Fig 3)

The numbers for this analysis were extracted directly from SaaS authorisation platform based on status of users' accounts, and directly from the list of registered users in CPMS included in the section Performance Indicators, based on the activity of the user. To increase the geographical coverage and therefore the accessibility to the CPMS ERN-EuroBloodNet opens a possibility for registration in CPMS not only for Members but also for Affiliated Partners with the same procedures and roles as full members, and Guest Experts from the Collaborating Centres (not officially recognized as Members) - these users have guest role with some administrative restrictions.

The action of increased geographical coverage will be continued and promoted in regards to the new Members Call 2019 resulting in the ongoing incorporation of additional 36 HCPs, with a potential new CPMS users representing lacking countries and giving the access to online and cross border medical consultation in the field of rare haematological diseases.

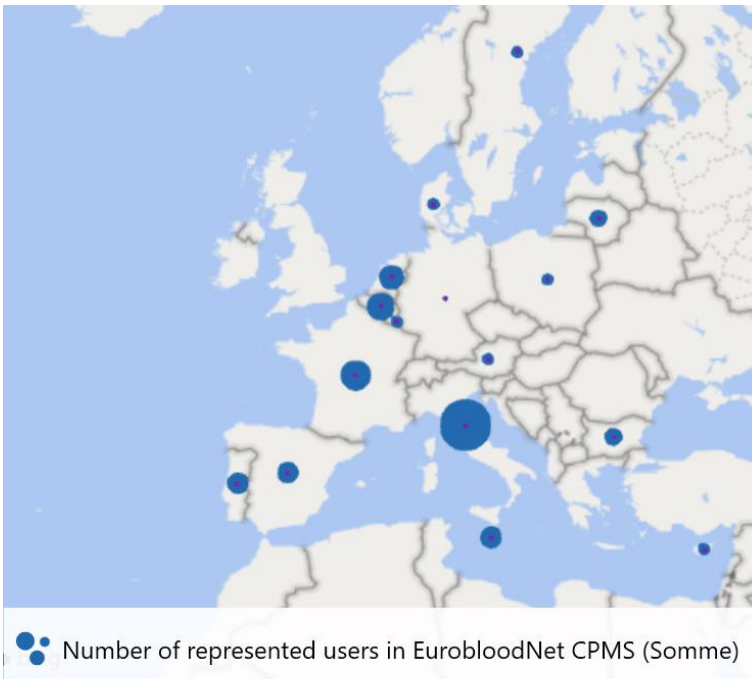

Fig 3. Number of represented users In EuroBloodNet CPMS

INCREASE IN DISEASE COVERAGE

In 2020 the pilot project to build a model for efficient cross border CPMS consultation was created in the frame of Connecting EuroBloodNet II, establishing the CPMS Virtual Board on Cutaneous Lymphoma.

The aim of it is to use the established strategy to promote CPMS Virtual Boards in other RHD networks to unable non-experts centers to submit cases to these Virtual Boards and to benefit their expertise when expertise Is lacking at their center and/or national level.

CPMS Virtual Board on Cutaneous Lymphoma allowed

- 14 experts in 7 MS were trained on the use of the CPMS
- Organization of 13 Virtual Board video meetings
- 9 cases enrolled in the CPMS with 8 outcome reports generated

CUSTOMIZATION OF THE CPMS

Actions have been undertaken to customize the CPMS and to adapt the platform to the specific needs and characteristics of Rare Haematological Diseases (RHDs).

A specific data set has been developed as a result from the gathering of feedback and input from all the CPMS actions performed from January 2019 to September 2019. After compiling all the feedback from experts, the variables for customization were organized in the most logical and comprehensive way while avoiding any redundant data in the datasets. Requests and recommendations are available in the Annex II of Measurable element 2.2.1 (ERN-EuroBloodNet CPMS needs). This outcome gathers the key parameters common to all the RHD for ensuring the adequate gathering of data for a) laboratory findings and b) follow up of the patients based on the RHD transversal needs, while sets the basis for a disease-specific customization. However, this data set is still pending for implementation from the European Commission.

A consultation form has been developed and used to complete the clinical patient data in more efficient and structured way. All these “best practices” reflect how experts from the network have tried to develop strategies to adapt the platform to their needs and to overcome the technical limitations faced within the CPMS.

Finally, based on the experience of its members and affiliated partners, the ERN-EuroBloodNet coordination has taken part to the discussion on the needs for improvement of the current platform, and the development of the new CPMS version with the European Commission and the IT team managing the tool. The CPMS Helpdesk has been regularly alerting the European Commission’s IT team about technical or complexity issues faced in the platform. Discussion have also started in 2021 regarding the need of a new CPMS version and the network has taken part actively to the discussion in order to reflect the specific demands of its members.

REGISTRIES

EUROPEAN RARE BLOOD DISORDERS PLATFORM (ENROL)

The European Rare Blood Disorders Platform (ENROL) is conceived as the ERN-EuroBloodNet umbrella platform for both new and already existing registries on Rare Hematological Diseases (RHDs) avoiding fragmentation of data by promoting the interoperability standards for patient registries in line with the EU RD Platform. ENROL is officially endorsed by the European Hematology Association (EHA).

ENROL's Principle is to maximize public benefit from data on RHDs opened-up through the platform with the only restriction needed to guarantee patient rights and confidentiality, in agreement with EU regulations for cross-border sharing of personal data. Accordingly, ENROL aims at EU-wide mapping of demographics, survival rates, diagnosis methods, genetic information, main clinical manifestations and treatments in order to obtain epidemiological figures and identify trial cohorts for basic and clinical research. To this aim, ENROL will connect and facilitate upgrading of existing RHD registries, while promoting the building of new ones when / where lacking.

ENROL dataset has been conceived in a bottom-up design for ensuring the capture of the common elements for rare diseases in line with the EU recommendations and with the EU RD platform, and the key features common for the whole spectrum for RHDs. Furthermore, the latest level can be stepped up for the definition of domain specific elements that support the in-depth analysis.

Accordingly ENROL common data set (CDS) contemplates the following three-levels-of-depth (Fig. 4)

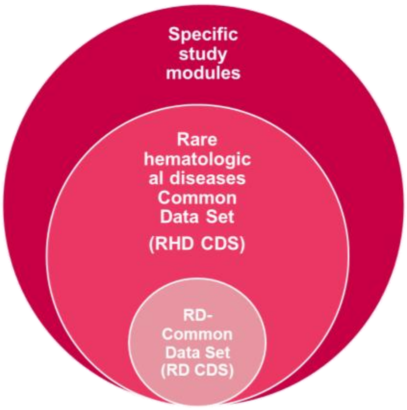

Fig 4. ENROL levels-of-depth CDS

1. Rare Diseases Common Data Set (RD-CDS) - set of mandatory parameters common to all RDs, essential for ensuring interoperability among European structures for RDs.

2. Rare Haematological Diseases Common Data Set (RHD-CDS) - set of mandatory parameters common to all RHDs, aiming to answer ENROL objectives

3. Domain specific RHD-CDS - set of mandatory/optional parameters common to concrete domains, e.g. Rare anaemia disorders, Blastic plasmacytoid dendritic cell neoplasm; or concretely developed for in depth analysis, ie. research oriented studies.

ENROL RHD-CDS has been defined with a total of 79 elements, including the 18 core data elements released by the EU-RD-Platform, and 61 additional elements common to all RHDs of high interest for epidemiological surveillance and generation of patients cohorts, ie. patients' stratification according to severity (i.e., chronic blood transfusion) and treatment options (i.e., Bone marrow transplant).

A key step in our process of defining the final ENROL dataset and making existing registries interoperable is using multiple codifications and international standards. Specifically, we used ICD10/11 and ORPHA classifications for the disease definition, HPO for the observed phenotype, LOINC codes for the laboratory parameters and NCIT for describing biomedical concepts and clinical symptoms. These actions enabled the detailed description of metadata and thus, setting the ground for developing a FAIR (i.e., Findable, Accessible, Interoperable and Reusable) platform for ENROL.

Next steps for promoting e-Health records in ERN-EuroBloodNet members include a mapping of availability of these elements in the EHR and use of related codifications e.g. ORPHA.

ERN-EUROBLOODNET COLLABORATIVE PLATFORM ON RED BLOOD CELL AND COVID-19 PATIENTS

Aiming to pool evidence for supporting daily medical practice on the clinical management of patients affected by red blood cell disorders (RBCDs) and COVID-19, ERN-EuroBloodNet launched a unique European collaborative platform resulting from a European effort to face this complicated situation.

The platform, opened to all the experts in Europe, has been recently upgraded with new data elements for its adaptation to the most recent updates on the field appropriate to draw conclusions from the data already registered as well as to continue learning from new registered patients. A patient representative has been involved in the Scientific Committee of the platform.

To December 2021 the platform counted with 373 patients. The abstract "ERN-EuroBloodNet European Registry of Patients Affected by Red Blood Cell Disorders and COVID-19" was presented during the American Society of Hematology (ASH) 2021 edition, and a peer review publication is under preparation.

On the other hand, the correct treatment and management of infection by Coronavirus (COVID-19) in patients affected by Rare Hematological Diseases was challenging given the rapid spread of the pandemic outbreak and limited literature at that moment. Accordingly, efforts were devoted by different initiatives for the generation of documents with indications for the clinical care of these patients.

A repository of key documents was produced with the participation of ERN-EuroBloodNet members and patients organizations. The repository was organized based on Subnetwork, language and target (health professionals or patients).

European
Reference
Network

https://ec.europa.eu/health/ern_en

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Hematological
Diseases (ERN EuroBloodNet)

www.eurobloodnet.eu

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