



6.1 ERN-EUROBLOODNET REPORT ON ACTIONS FOR THE PROMOTION OF CONTINUING MEDICAL EDUCATION ON RHD

ERN-EuroBloodNet

European Reference Network on Rare Hematological Diseases

EUROPEAN REFERENCE NETWORKS
FOR RARE, LOW PREVALENCE AND COMPLEX DISEASES

Share. Care. Cure.



Co-funded by
the Health Programme
of the European Union

Disclaimer:

The content of this deliverable represents the views of the authors only and it is their sole responsibility; it cannot be considered to reflect the views of the European Commission and/or the Consumers, Health, Agriculture and Food Executive Agency (Chafea) or any other body of the European Union. The European Commission and the Agency do not accept any responsibility for use that may be made of the information it contains.

DOCUMENT INFORMATION

6.1 ERN-EUROBLOODNET REPORT ON ACTIONS FOR THE PROMOTION OF CONTINUING MEDICAL EDUCATION ON RHD

Report document

ERN: **ERN-EuroBloodNet (European Reference Network on Rare Hematological Diseases)**

Call: **HP-ERN-SGA-2018**

Type of action: **HP-SGA-PJ**

Authors:

Mariangela Pellegrini – ERN-EuroBloodnet Manager

Victoria Gutierrez Valle – ERN-EuroBloodNet Dissemination & IT Manager

Maria del Mar Mañú Pereira – ERN-EuroBloodNet Scientific Director

Patricia Aguilar-Martínez – WP5 non-Oncological coordinator

Dominique Bron – WP5 Oncological coordinator

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 activities implemented in collaboration with educational bodies (EHA and ESH) for the promotion of CME on RHD.

Publication Date

05/05/2020

TABLE OF CONTENTS

1. Introduction

- 1.1 Rationale and background
- 1.2 Objective and structure

2. Educational actions implemented

- 2.1 Webinar programs for health professionals and patients organisations on Rare Hematological Diseases
 - 2.1.1 “EuroBloodNet Thursdays Webinars”. Transversal program for health professional covering all RHDs
 - 2.1.2 Comprehensive Webinar program on Cutaneous Lymphoma for health professionals
 - 2.1.3 Comprehensive Webinar program on Cutaneous Lymphoma for patients associations
- 2.2 Preceptorships for health professionals carried out on HCPs sites and coordinated by ERN-EuroBloodNet experts
 - 2.2.1 Results from Preceptorship on AA and PNH
 - 2.2.2 2020 ERN-EuroBloodNet Preceptorship program on SCD
- 2.3 Establishment of the European Network of Sickle Cell Disease Patients Organisations
- 2.4 Sickle Cell Disease Research Prioritisation Workshop at ASCAT 2019

3. Conclusions

4. Annexes

- Annex I List of topics for Thursdays webinars
- Annex II ASCAT Agenda
- Annex III ASCAT Poster Results
- Annex IV ASCAT questionnaire results

1. INTRODUCTION

One of the ERN's most ambitious objectives, given that they deal with rare diseases (RD) is the promotion of continuing medical education (CME) for both professionals and patients specifically targeting these conditions.

The first reason that makes this objective as an ambitious one is the fact that it is very difficult to produce and/or find educational material because of the low prevalence of rare diseases. This situation is due to different conjoint causes:

- 1) RD are not so often represented at scientific congresses sessions.
- 2) It is difficult to develop guidelines or launch clinical trials, due to the low number of patients affected by RD.
- 3) It is difficult to identify patients' cohorts' cause of the fragmented and not harmonized presence of RD registries on the European territories.

The same complex situation affects the possibility for the RD patients to receive an adequate education with respect to the pathology they suffer from. Only when a disease is covered by an experienced patient association, there is the possibility to find highly quality validated educational documents and get the patient empowered.

The educational opportunities for a physician interested in training in the clinical field of Rare Hematological Disorders (RHD) are not many nor easily accessible. Such CME resources are even scarcer if the field of interest belongs to benign hematological disorders, or to ultra-rare conditions both oncological and non-oncological. These courses are few and, where existing, very expensive since the available resources of experts from which to draw knowledge are not extensive, making necessary the co-participation of a board of international experts and the use telematics tools (online courses, webcasting, etc).

As stated in the "Deliverable 5.1 Report on the educational gaps" of the second year of the network (HP-ERN-SGA-2017-811641), the ERN-EuroBloodNet Transversal Field of Action on CME, coordinated by Dominique Bron, as oncological representative, Patricia Aguilar Martinez as non oncological representative and Jan Geissler as ePAG representative for the Lymphoid disorders, focussed on the on the identification of the educational gaps existing in Europe with respect to RHD, for patients and health professional. Also, a collaboration among ERN-EuroBloodNet and two European well-recognized educational bodies, European Hematology Association (EHA) and European School of Hematology (ESH), was established in order to strength collaboration

In this context, based on the results in the previous period, ERN-EuroBloodNet specific CME objective of third year has been to build blended educational programs (on-site and online) with innovative educational contents covering existing gaps.

2. OBJECTIVES

Taking into consideration the results of the identified gaps expressed in the "Deliverable 5.1 Report of educational gaps" in the previous period of network implementation, the following objectives were identified for this action:

- 1) To disseminate most innovative topics among health professionals in order to gain insight into the last cutting-edge advances in the field of RHDs, focusing on: Very rare diseases, Complex disorders, Highly specialized procedures, Implementation of guidelines.
- 2) To promote very innovative topics among health professionals and patients organisations in order to give visibility to the last cutting-edge advances related to a specific ultra-rare disorder.
- 3) To organise on-site training (preceptorships) within highly specialized ERN-EuroBloodNet Healthcare Provider (HCPs) and coordinated by ERN experts. Those training are not only theory and scientific background oriented but they experience also the insight into practical management of patients with rare hematological diseases at the highly specialized RHD centres - from the early diagnosis to treatment of complicated cases.

This deliverable describes all the ERN-EuroBloodNet actions implemented by the Network during the third year period for addressing the identified educational gaps described in the "Deliverable 5.1 report on educational gaps" of 2019.

The different actions reported include the following sections:

- Rationale
- Objective
- Methods
- Results
- Next steps

3. EDUCATIONAL ACTIONS IMPLEMENTED

2.1 WEBINAR PROGRAMS FOR HEALTH PROFESSIONALS AND PATIENTS ORGANISATIONS ON RARE HEMATOLOGICAL DISEASES

Rationale

ERN-EuroBloodNet is aware of the high impact that the online activities have nowadays, especially in the area of education. Topic specific recorded video sessions provided by experts in the field allow health professionals and patients to learn highly specialized knowledge without the need to travel and whenever they wish. In addition, topic focused webinars allow to tackle questions gathered from the audience in real time, providing the perfect environment to benefit from the most outstanding experts in the field.

In the second instance, ERN-EuroBloodNet aims to take advantage of the educational material gathered through webinar, putting it on an online repository, in order to make it available to health professionals and patients community not only at the time of the online lesson but also in the years to come. In fact the material will be gathered on an eLearning platform which is described in the next paragraphs.

ERN-EuroBloodNet Coordination team together with TFA coordinators of CME has planned the strategy for tailoring the Webinar programs for health professional. In this context EuroBloodNet Thursdays Webinars have been established for a transversal educational program dedicated to all RHDs (see section 2.1.1).

In addition, efforts are being devoted for the definition of comprehensive programs compound on several sessions on very rare diseases given the higher necessity for in-depth knowledge sharing. First disease identified is Cutaneous Lymphoma (CL), where a task force of health professionals and patients advocates (including Coalition of patients organizations) have been identified willing to establish:

- Comprehensive program on CL for health professionals(see section 2.1.2)
- Comprehensive program on CL for patients' organizations (see section 2.1.3), in collaboration with EURORDIS, and Lymphoma Coalition Europe and Cutaneous Lymphoma Foundation.

The three webinar programs strategy were presented during the Board of Network meeting held in November 2019 in Barcelona and have been approved in unanimous by the Board.

Methods

Webinars are online educational activities conducted using the Webex Platform provided to ERN-EuroBloodNet by the European Commission. It allows full audio and visual communication, as well as interaction between webinar's speaker and participants.

Webinars are led by the speaker with the visual aid of a Power Point presentation and recorded by the coordination team. The mp4 audio got by the session and the power point presentation will be uploaded and made available on the ERN-EuroBloodNet webpage.

At the end of the Health professional webinar program a slide in the Power Point Presentation mentions the upcoming educative events organised by ESH with respect to the topic of the session. Whereas at the end of the Patients webinar program a Power Point Presentation slide will mention the upcoming events organised by Patients associations, Networks, etc. with respect to the topic of the session.

According to the directive of the GDPR, speakers are requested to sign a presentation publishing agreement to authorize the publication of the webinar recorded together with the slides presentation on the ERN-EuroBloodNet website. Also, webinar hearers are requested to sign consent through a checkbox in the registration form on ERN-EuroBloodNet website, by joining the webinar the participant accepts the possibility of being recorded if comments/questions are made and understand that those will be publicly available at the website.

2.1.1 "EUROBLOODNET THURSDAYS WEBINARS". TRANSVERSAL PROGRAM FOR HEALTH PROFESSIONAL COVERING ALL RHDs

Objective

Main objective of the Webinar "EuroBloodNet Thursdays Webinars" is promoting the interest on very innovative topics in order to stress among health professionals the cutting-edge advances in the field of Rare Hematological Diseases. Webinar are mainly addressed on very rare diseases, complex disorders, highly specialized procedures and implementation of guidelines. Apart from defining a focus of interest that could be neglected because too specialized or dedicated to ultra-rare diseases, webinars gives also visibility to upcoming educative events organized by EHA and ESH with respect to each session's topic.

Webinar topics

First topics list, based on cutting-edge research results obtained within the ERN-EuroBloodNet members, have been shaped by ERN-EuroBloodNet Coordination Team and TFA CME coordinators by analysing the results from the survey “ERN-EuroBloodNet questionnaire on CME” as described in the “Deliverable 5.1 Report of educational gaps” of the previous year of network implementation, and the List of international guidelines and recommendations as explained in “Deliverable 4.1 Report on the comprehensive public database of reliable guidelines” also from that period. The cross-checking analysis has led on one hand, to understand the educational needs and expectations of the centres of excellence and on the other hand to exploit, within the network, material and knowledge already available.

To this first list, a second one has been edit by consulting the ERN-EuroBloodNet Board of Network in November 2019 in Barcelona. 23 new topics have been identified. Full list including topic and subnetwork are available in Annex I List of topics for Thursdays webinars.

Webinars target

Webinars are opened to every haematologist, paediatrician, or other health care providers interested in the topic, within and outside the ERN-EuroBloodNet.

Webinars speakers

Speakers are identified among ERN-EuroBloodNet members.

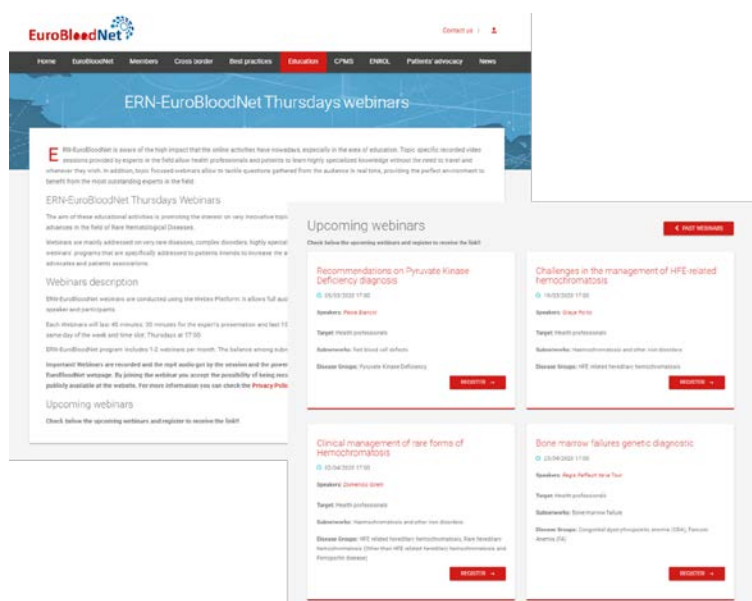
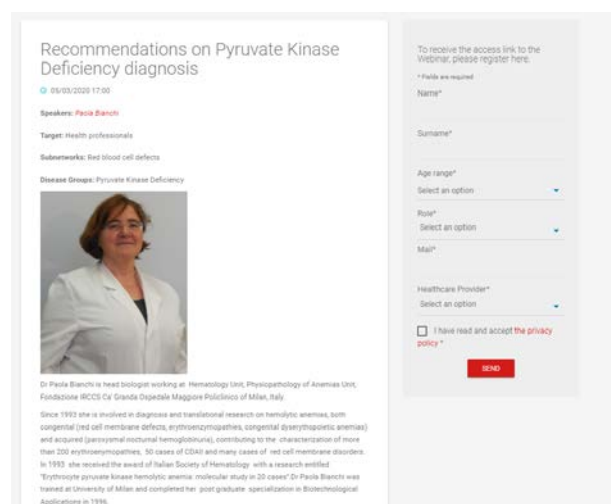
Method for EuroBloodNet Thursdays Webinar session

Each Webinar lasts 45 minutes: 30 minutes for the expert’s presentation and last 15 minutes for hearers’ questions. In addition, they are always held in the same day of the week and time slot (Thursdays at 17:00). ERN-EuroBloodNet aims to implement 2 webinars per month. The balance among subnetworks will be ensured, holding 2-4 webinars/year/subnetwork.

ERN-EuroBloodNet Thursdays webinars program were officially launched in January 2020, with the release of a [Webinars dedicated section](#) at ERN-EuroBloodNet website. Section includes a brief introduction and aims of the program and methods, together with a) specific informative boxes for each of the upcoming webinars and direct link for registration and b) Link to past webinars.

Registration form includes the following fields mandatory to receive the link for connection:

- Name
- Surname
- Age range
- Role
- Mail
- Healthcare Provider (list of ERN-EuroBloodNet members and "Other" option for free text)
- Checkbox "I have read and accept the privacy policy"

[First program has been established from February 2020 to September 2020](#) as follows, and is in continuous updated as dates are confirmed by the speakers:

- 06 February 2020, Prof. Nicolas Boissel, Management of relapsed/refractory Acute Lymphoblastic Leukaemia in adult in adult
- 13 February 2020, Prof Eduard Van Beers, Pyruvate kinase deficiency Clinical management
- 05 March 2020, Dr Paola Bianchi, Recommendations on Pyruvate kinase deficiency diagnosis
- 19 March 2020, Prof Graça Porto, Challenges in the management of HFE-related hemochromatosis
- 02 April 2020, Prof Domenico Girelli, Clinical management of rare forms of Hemochromatosis
- 23 April 2020, Prof Regis Peffault de la Tour, Bone marrow failures genetic diagnostic
- 14 May 2020, prof Dominique Bron, How to approach older patient with Chronic Lymphocytic Leukemia
- 28 May 2020, prof Martine Bagot, Treatment of Cutaneous lymphomas
- 25 June 2020, Dr Roberta Russo, Congenital dyserythropoietic anemias
- 9th July 2020, prof Uwe Platzbecker, Treatment of anemia of low risk Myelodysplastic Syndrome
- 17 September 2020, Prof Patricia Aguilar Martinez, Genetic counselling of Hemophilia
- 24 September 2020, Dr Immacolata Andolfo, Hereditary Stomatocytosis

As result, the number of sessions by subnetworks by the moment is represented in Fig 1.

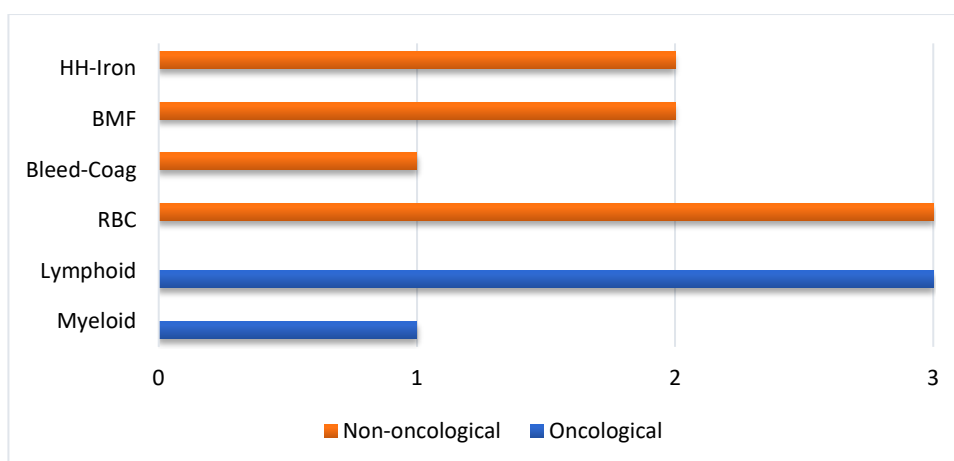


Fig. 1 Number of webinars sessions by subnetworks already scheduled (HH-Iron: Hemochromatosis and other rare genetic disorders of iron metabolism and heme synthesis, BMF: Bone marrow failures, Bleed-Coag: bleeding-coagulation disorders, RBC: Red blood cell disorders)

A brief anonymous survey is conducted among the attendants following the end of the Webinar, which includes the following questions:

From 1 (Not at all) to 5 (Absolutely yes!), how would you rate the webinar?

- Has the webinar been useful to increase your knowledge on the area?
- Will you translate the knowledge learned into the management of patients?
- Would you recommend the webinar to other colleagues?
- Would you suggest any other topic you would like to have as a Webinar session? (free text)

Results from February Webinars

Two first webinars have already been held during the month of February.

- 06 February 2020, Prof. Nicolas Boissel, Management of relapsed/refractory Acute Lymphoblastic Leukaemia (ALL) in adult -11 attendants (3 of them linked to non-EuroBloodNet members)
- 13 February 2020, Prof Eduard Van Beers, Pyruvate Kinase Deficiency (PKD) Clinical management - 9 attendants (all of them from EuroBloodNet members)

Figure 2. represents the country coverage of the attendants to both webinars. A higher interest can be appreciated on for the red blood cell related webinar (PKD Clinical management) in those countries where traditionally are higher prevalence of these disorders (naming haemoglobinopathies), as Italy, United Kingdom or France.

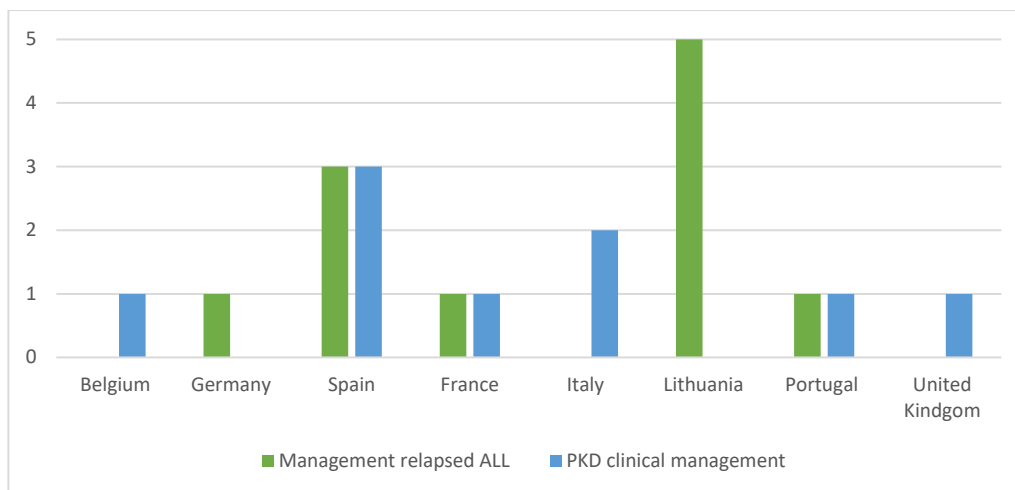


Fig. 2 Country coverage of the attendants to the ERN-EuroBloodNet Thursdays webinars on February 2020

Fig. 3 Includes the roles of the attendants. Most of the attendants to the webinars are Hematologist (65%), followed by pediatricians (25%). The percentage of pediatricians is expected to be increased as a higher number of webinars for the non-oncological disorders are held, since the oncological coverage of the network is only for adults.

Attending to the age range, the higher representation at the webinars are 20-40 age range. Fig. 4 details the numbers of attendants per age range.

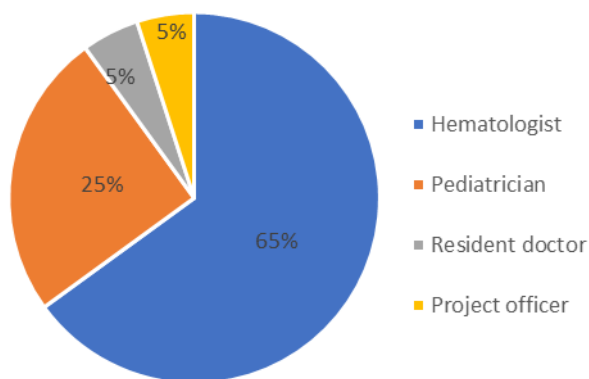


Fig. 3 Roles of attendants (%)

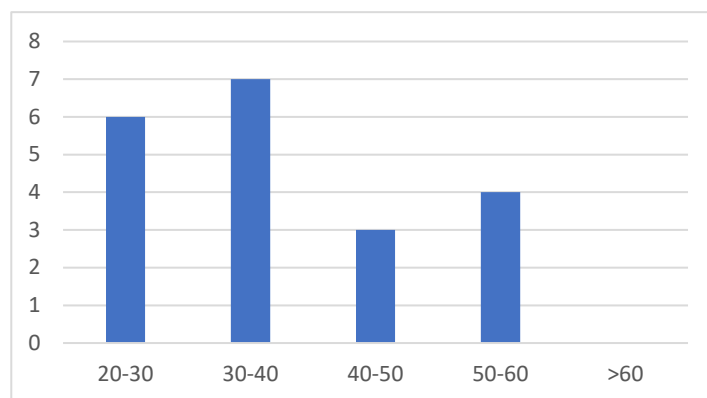


Fig. 4 Number of attendants per age range

Attending to the results of the satisfaction survey, a total of 11 answers were received (7 from ALL webinar, and 4 from PKD webinar). The media gathered from answers to the questions rated from 1 (Not at all) to 5 (Absolutely yes!) are compiled in Fig. 5. Feedback in general terms was excellent. A total of three "4" rates were received, two of them in the translation of knowledge into management of patients (one for each of the webinars held), and one in the usefulness to increase the knowledge in the ALL webinar.

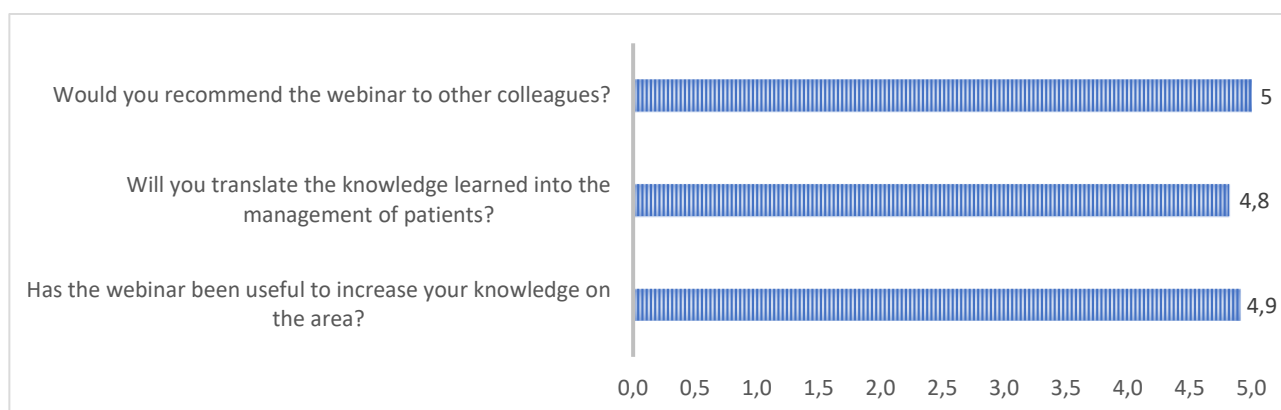


Fig. 5. Media of answers from the satisfaction questionnaire (rated 1 (Not at all) to 5 (Absolutely yes!))

Also, the following suggestions were received for upcoming webinars:

- NK/T cell lymphoma
- Monoclonal gammopathy of renal significance
- Management of CART toxicity

Next steps

1. Enhance the dissemination of the Program aiming to increase the number of participants by the diffusion among National Scientific Societies contact persons identified.
2. Creation of a repository of questions rose each session.
3. Continue programming the calendar to organize further educational sessions by taking into consideration the 23 topics proposed by the Board of Network.

2.1.2 COMPREHENSIVE WEBINAR PROGRAM ON CUTANEOUS LYMPHOMA FOR HEALTH PROFESSIONALS

Objective

Main objective is to disseminate very innovative topics among health professionals in order to give visibility to the last cutting-edge advances in the field of Cutaneous Lymphoma. This objective responds not only to the need for in-depth knowledge sharing by international experts of this rare malignancy, but also to the need to create a repository of comprehensive educational material on all types of Cutaneous Lymphoma. In fact there are several differentiations in Cutaneous Lymphoma sub-categories that require a specific educational knowledge from a clinical, pathological, etiological and phenotypic point of view.

Finally this educational program would clarify the role of hematologists and dermatologists in the clinical managing of Cutaneous Lymphoma patients as this disease requires a multidisciplinary approach because it manifests itself on the skin level.

Webinars target

Webinars will be opened to every haematologist, paediatrician, or other health care providers interested in the topic.

Webinars speakers

Speakers are identified among ERN-EuroBloodNet members.

Webinars sessions

Each Webinars will last 45 minutes: 30 minutes for the expert's presentation and last 15 minutes for hearers' questions. In addition, they will be held always in the same day of the week and time slot (each Monday at 5 pm). ERN-EuroBloodNet aims to implement 1 webinar per month.

Program

The program has been drafted by ERN-EuroBloodNet Coordination team with TFA CME coordinators, lymphoma subnetwork ePAGs and Cutaneous Lymphoma Task Force (Cutaneous Lymphoma experts + European Lymphoma Coalition).

Program will start in May 2020 and end in December 2020. The calendar is under setting. To date of this deliverable, 8 educational sessions have been identified:

1. Introduction to CL, prof Rein Willemze
2. Mycosis Fungoides, prof Maarten Vermeer and prof Antonio Cozzio
3. Sézary Syndrome, prof Martine Bagot and prof Pietro Quaglino
4. CD30+lymphoproliferations, prof Koen Quint and prof Werner Kempf
5. Aggressive T-cell lymphomas, prof Sean Whittaker and prof Pietro Quaglino
6. PCFCL and PCMZL, prof Pablo Ortiz and prof Emmanuela Guedenova
7. PCLBCL, Leg type, prof Marie Beylot Barry and prof Anne Roos
8. New therapeutic developments, prof Julia Scarisbrick and prof Rudy Stadler

An official request to the European Board for Accreditation in Hematology (EBAH) will be submitted in order to be recognized as a CME accredited program.

Next steps

1. Finalize the agreements for the definition of program topics, speakers and dates.
2. Official request for EBAH CME accreditation
3. Implementation of dedicated section at ERN-EuroBloodNet website
4. Repository of questions rose each session
5. Analyse the expansion of this approach to other very rare diseases. Second target already identified: Haemochromatosis and other very rare iron disorders.

2.1.3 COMPREHENSIVE WEBINAR PROGRAM ON CUTANEOUS LYMPHOMA FOR PATIENTS ASSOCIATIONS

Objective

Main objective is promoting the awareness on Cutaneous Lymphoma among patients' advocates and patients association (English speakers). Program will give general information about CL as an introduction to the program; but it will be also disease oriented. This means that it will take into account the specificity of each kind of Cutaneous Lymphoma. The idea is that the webinar's hearer should understand diagnosis, treatments and management of specific type of Cutaneous Lymphoma to be better prepared to support within his/her patients' organization newly and already diagnosed patients. Program should be a comprehensive one, made by several sessions. It will be held once per month at a fixed day and timeslot.

Webinar target

Webinars will be opened to patient's advocates, but also to relative patients and caregiver interested in the topic. Despite the program is open to every interested person, the target for structuring the program should be: expert patients and patients associations. So, the webinars can get more technical; introduce the scientific and clinical aspects of the Cutaneous Lymphoma.

Webinars speakers

Speakers and topics are ordinarily identified by the Cutaneous Lymphoma Task Force working on Patients Organisations Webinars: ERN-EuroBloodNet Coordination Team, CL experts, patients associations, Europe Lymphoma Coalition and EURORDIS. Each webinar session will be coordinated by a duo: Health professional and a patient.

Draft of the programs

The program will be reviewed in June, after the release of the results of the survey conducted by Lymphoma Coalition Europe "[The 2020 Lymphoma Coalition Global Patient Survey on Lymphomas and CLL](#) " whose theme focuses precisely on the educational needs and awareness of the patient. The survey is available in 19 languages. The information gathered will help the Lymphoma Coalition to be better equipped to advocate for change that will improve care globally and within countries, as well as provide you with the support patients may need locally.

Lymphoma Coalition Europe will share with us the result allowing us to build a truly tailored program on the real, practical and concrete needs of the patient. The ERN-EuroBloodNet Webinar comprehensive program on Cutaneous Lymphoma for patients'

organisations will be tailored and based on the 2020 Lymphoma Coalition Global Patient Survey on Lymphomas and CLL for guarantee that needs expressed by patients and caregivers in the survey will be listened and tackled.

The final proposal of the program will be submitted to a board of lymphoma experts' patients for validating it.

The first webinar will be just be focused on an overall introduction to all the subtypes and specific issues link to these types of very rare lymphomas.

Disease oriented

1. Introduction CL
2. Mycosis Fungoides
3. Sézary Syndrome
4. CD30+ LPD
5. Aggressive TCL
6. PCFCL and PCMZL
7. PCLBCL, LT

General

1. Presentation of the subtype
2. Introduction health professionals (dermatologist, hematologist, pathologist, radiation oncologist)
3. Introduction diagnostic procedures (biopsy, CT-scan, blood)
4. Skin care and Itch
5. Treatments:
 - a) Topic treatments *
 - b) Radiotherapy (how can I prepare what can I expect?)*
 - c) Systemic treatments (how can I prepare what can I expect?)*
 - a. Oral*
 - b. Infusion*
6. Everyday:
 - a) Nutrition
 - b) Sexuality
 - c) Other tips and tricks

* If applicable

The CL Task Force has also suggested to insert before each session, as an introduction to the related topic, an interview with a patient, for example under the format of pre-recorded video of less than 5 minutes.

Next steps

1. Program will be reviewed in June, after the results of the Survey launched by Lymphoma Coalition Europe on Educational CL patients' needs, together with the establishment of a duo of physicians/experts patients as identified speakers of the webinar program and a board of patients for validates the webinar program.
2. Disseminate and launch the program.
3. Evaluate if the webinar program could be addressed to patients at national level, or if it is too technical for a non-expert patient.

2.2 PRECEPTORSHIPS FOR HEALTH PROFESSIONALS CARRIED OUT ON HCPs SITES AND COORDINATED BY ERN-EUROBLOODNET EXPERTS

2.2.1 RESULTS FROM PRECEPTORSHIP ON AA AND PNH

Rationale

As described in “Deliverable 5.1 Report on Educational Gaps” of previous year of network implementation, ERN-EuroBloodNet implementation Paroxysmal nocturnal hemoglobinuria (PNH) and Aplastic Anemia (AA) were identified as one are to be benefited from the organization of short stays due to the lack of expertise in its diagnosis, leading potentially to a worsen of prognosis on the health’s patient.

Objective

The priority aim of preceptorship on PNH and AA is to provide applicants with the fundamental tools for a correct diagnostic and treatment approach to bone marrow failures, including AA and PNH in children, adolescents and adult patients.

Methods

ERN-EuroBloodNet has endorsed [three preceptorships held in ERN-EuroBloodNet highly specialized centers for AA and PNH](#) on highly specialized centers among EuroBloodNet Health Care Providers through 3-4 short stays and different programs on specific topics of expertise of the center and multidisciplinary teams:

- 1) Preceptorship 1 – Inherited and acquired bone marrow failures including AA and PNH in adult and pediatric patients
 - From 13/01/2020 to 16/01/2020 (4 days)
 - Host Center: AP-HP, Hôpital Saint-Louis, Paris, France
 - Coordinated by: Prof Regis Peffault de la Tour
- 2) Preceptorship 2 - Acquired bone marrow failure syndromes: aplastic anemia and paroxysmal nocturnal hemoglobinuria from the bench to the bedside
 - From 03/02/2020 to 5/02/2020 (3 days)
 - Host Center: Federico II University of Naples, Italy
 - Coordinated by: Prof. Antonio Risitano
- 3) Preceptorship 3 - Inherited, acquired and overlap bone marrow failures in children and adolescents
 - From 17/02/2020 to 20/02/2020 (4 days)
 - Host Center: G. Gaslini Research Childrens' Hospital, Genova, Italy
 - Coordinated by: Dr. Carlo Dufour

Results

The Preceptorship program for AA and PNH has gathered a total of 13 Participants from 8 Member States (Czech Republic, Germany, Italy, Lithuania, Netherlands, Portugal, Spain and United Kingdom) attending to:

- 3 Participants selected for Preceptorship 1 (Paris)
- 5 Participants selected for Preceptorship 2 (Naples)
- 5 Participants selected for Preceptorship 3 (Genova)

Feedback reported for both, participants and professionals hosting, have reported wonderful comments on the experience as:

- This very first EuroBloodNet preceptorship surely exceeded my expectations. The majority of continuing education events I have ever attended were much more theoretically orientated. This preceptorship was totally different. It offered to us (a small group of three) not only up-to-date theoretical and scientific background but also the insight into practical management of patients.
- The preceptorship was a fantastic learning opportunity and possibly the only guarantee I've had in my career of being guided by world experts with every conversation. The teaching was perfect, the group size was ideal and the layout of the day worked well for us
- This was more PNH patients than I have ever seen. In fact if the preceptorship were only 2 days and one morning was spent in clinic this would be good enough as the learning value of that clinic was so high!
- A deep focus on diagnostic tools and treating strategies let to understand the challenges of bone marrow disorders on overlapping age of children and adult.

- A highly motivated participants in a small group allowed to individualize the learning and to achieve a maximum result of education as well as to create the bridge for the future cooperation.

The comments received from the first preceptorship hosted by AP-HP, Hôpital Saint-Louis, Paris, France under the coordination of Prof Regis Peffault de la Tour during 13-16 January 2020 were compiled and published at the piece of news:

- ["The majority of continuing education events I have ever attended were much more theoretically orientated. EuroBloodNet preceptorship was totally different." Read the testimonies of the first PNH and AA program!](#)

Finally, the three ERN-EuroBloodNet Preceptorships have been successfully accredited by EBAH (European Board for Accreditation in Hematology). Each preceptorship has obtained 18 credits.

2.2.2 2020 ERN-EUROBLOODNET PRECEPTORSHIP PROGRAM ON SCD

Rationale

In order to improve cutting-edge knowledge on Rare Hematological Diseases (RHD), ERN-EuroBloodNet has identified across Europe the healthcare areas including highly specialized procedures requiring preceptorship for the acquisition of medical expertise. Second interventional healthcare area identified is: Sickle Cell Disease.

Due to a number of factors, Sickle Cell Disease is on the rise Europe and therefore the need to increase expertise in clinicians responsible for patient care in areas of high and low prevalence. A number of countries like the UK and France traditionally have high SCD prevalence in the range of 14-20,000 people living with the disorders respectively, while others like Spain, Italy and Germany have seen a more steep rise in the affected people in their midst recently. All patients with SCD irrespective of where they live are entitled to receive high quality care that is able to meet their needs for emergency treatment, surveillance and rehabilitation where necessary. This is the driving force for the current service review across Europe, leading to the emergence of Haemoglobinopathy Coordinating Centres, Specialist Treatment Centres and National Clinical Reference Group to ensure equity of provision and high quality care.

Objective

Main objective in endorsing Preceptorship program on SCD is to increase awareness of current management of sickle cell disease, delivering excellence in patient care education, clinical audit and stimulate research interests.

In general ERN-EuroBloodNet Preceptorship objectives are:

- To train medical residents in hematology in order to improve the speed and the efficiency of patients 'care and cure.
- To inform young physicians about the effective possibility for a RHD patient to be followed in highly quality health services in Europe.
- To foster cross-border collaborations between medical residents in hematology.
- To promote among young physicians the best practices in diagnosis and safe clinical care.
- To give students through an accredited program an opportunity for broad experience and clinical training

Methods

Preceptorships are carried out on HCPs sites that are excellence centre in the ERN-EuroBloodNet and they are coordinated by ERN-EuroBloodNet experts.

A call for participants will be launched and candidates will be chosen by a jury according to: CV+ cover letter + CBH collaboration needs + follow-up with education projects and activities on medical education.

Preceptorship structure will be:

- From 2 to 3 days of intensive program.
- From 5 to 10 participants per host center.
- Preceptorships are based on clinical cases.
- EuroBloodNet experts have communicated among them in order to present a common program at national level.
- Several format: formal lectures, informal discussion, case study discussion, and direct tutorial activity (in the clinic and/or in a lab).
- Each candidate can participate only to one preceptorship program.

Results

1. Preceptorship

- Host Center: ASST-Monza, S. Gerardo Hospital, Fondazione of Milano-Bicocca, Pediatric and Adult Units for hereditary anemias. Monza, Italy
- Coordinated by: prof Alberto Piperno
- Topic: Sickle cell disease: from prenatal/neonatal screening, prenatal diagnosis, pediatric follow-up to adult management, pregnancy management, management of complications and treatment approaches

2. Preceptorship

- Host Center: AP-HP Hôpital Universitaire Necker, Enfants Malades, Paris, France
- Coordinated by: prof Mariane de Montalembert
- Topic: Sickle Cell Disease from the bench to the bedside, children and transition.

3. Preceptorship

- Host Center: Evelina London Children's Hospital, Guy's and St Thomas NHS Trust (GSTT), London, UK
- Coordinated by: Dr Baba Inusa
- Topic: Comprehensive care for SCD in the era of post-hydroxycarbamide therapies and increasing access to emerging new therapies: Ensuring effective education and shared decision making with patients and their families.

4. Preceptorship

- Host Center: CHU de Pointe-à-Pitre/Abymes, Pointe à Pitre, Guadeloupe, France
- Coordinated by: Prof Maryse Etienne Julan
- Topic: Sharing expertise among overseas European Countries. Sickle cell disease: from prenatal/neonatal screening, prenatal diagnosis, pediatric follow-up to adult management, pregnancy management, management of main acute complications, prevention of some chronic complications and treatment approaches

5. Preceptorship

- Host Center: Hospital Universitari Vall d'Hebron, Barcelona, Spain
- Coordinated by: Prof Cristina Diaz de Heredia
- Topic: Sickle Cell Disease from the bench to the bedside, children and transition.

An official request to the European Board for Accreditation in Hematology (EBAH) will be submitted in order to be recognized the preceptorship as an accredited program.

Next steps

1. Ultimate the program and launch the call for participants.
2. Other clinical identified areas of intervention are: Willebrand disease, Cutaneous Lymphoma
3. Request EBAH accreditation.

2.3 ESTABLISHMENT OF THE EUROPEAN NETWORK OF SICKLE CELL DISEASE PATIENTS ORGANISATIONS

Rationale

The European Reference Network on rare hematological diseases, ERN-EuroBloodNet, in collaboration with Eurordis, is establishing the European Network of Sickle Cell Disease (SCD) Patients Organizations: a bottom-up umbrella network of national and local organizations of SCD patients' advocates, whose objective is to give a strong representation of the disease and make its needs visible at European level.

SCD is an inherited disorder of the red blood cells. Being a lifelong chronic condition, SCD can lead to disability or even premature death in its severe forms. Up to now, SCD is one of the few rare anemias that do not have, in every country in Europe, an adequate educational contribution for patient's awareness on daily management of the disease. SCD is indeed traditionally endemic in African and Middle East countries but their frequency has increased recently in Europe due to migration and mobility flows. So, the patients' educational needs are not explicitly covered, since the awareness level of the disease and the presence of the patients' organizations at the national level are extremely varied from country to country. This is even more reinforced by the fact that those patients often face integration difficulties, being a population largely coming from third countries, with less facility to access care services.

Objective

The structuring of the European Network of SCD patients' organizations aims to create, within the ERN-EuroBloodNet, the conditions for the development of a strategic European patients' group.

The European Network of Sickle Cell Disease Patients' Organisations aims to provide a centralized European point of contact for patients and caregivers while supporting the synergy among existing national support groups. Moreover among all the spokespersons, one representative will be formed as ePAG for ERN EuroBloodNet.

The final aim of the European Network will be the on support policies on rare diseases to be implemented to ensure that patients' needs are at the heart of health and research policies.

Methods

The concrete idea is to establish a bottom-up network of national and local sickle cell advocate organizations. Each European country could have two or more spokespersons representing all national patient organizations. The recommendation is to have one national representative and one substitute. Among all the spokespersons, one representative would become the ePAG of ERN EuroBloodNet.

ERN-EuroBloodNet is therefore working to organize events that encourage the meeting of SCD patients and their families at the local level, in the countries where this problem is most prevalent. The ERN is also promoting educational activities addressed to SCD patients in order to encourage their formation as advocate and expert patients. This is how ERN-EuroBloodNet is providing a representation of SCD European community able to make manifest patients' needs and facilitate access to care.

A total of 3 National meetings (Italy, France, Spain&Portugal) and 1 informative national meeting (Belgium) has been held during third year of the ERN-EuroBloodNet.

1. First national meeting in Milan, the 29th of March 2019
2. Informative National Meeting in Bruxelles, the 19th of June 2019. ERN-EuroBloodNet presenting the project during the international SCD day in Belgium
3. Third National Meeting, in Paris, the 29th of June 2019
4. Fourth National Meeting, in Madrid, gathering Spanish and Portuguese patients, 18th February 2020

Next steps

- Organizing further national meetings for having a solid European patient's representation, ie. The Netherlands and Ireland
- Set the first Board meeting where the board of the European Network of SCD patients organizations will gather together for setting first actions and validate Network characteristics and chart of conduit.

2.4 SICKLE CELL DISEASE RESEARCH PRIORITISATION WORKSHOP AT ASCAT 2019

Rationale

As already mentioned in the previous paragraph, Sickle Cell Disease (SCD) is an inherited disorder of the red blood cells, a lifelong chronic condition that can lead to disability or even premature death. ERN-EuroBloodNet is promoting educational activities addressed at people living with SCD in order to encourage their training and develop their skills as advocate and expert patients. ERN-EuroBloodNet provides a representative SCD community able to address patients' needs. Using this platform priorities address access to care.

Objective

The SCD Research Prioritisation Workshop at the Annual Scientific Conference on Sickle Cell and Thalassaemia (ASCAT) was a joint project of the ERN-EuroBloodNet together with ASCAT members aiming to

- Train patients to Public Patient Involvement in Research
- Train health professionals on a patient research center approach

Methods

Workshop was held 20th October 2019 ("Annex II ASCAT Agenda") following an adapted methodology from the James Lind Alliance to gain a valid consensus. The workshop was moderated by three experienced facilitators. The workshop was moderated by three experienced facilitators from Oxford University Hospitals: Dr Noémi Roy, Sandy Hayes and Dr Catriona Gilmour-Hamilton. The first step was to create a patient forum with at least 20 patients coming from EU countries to share experiences, built cross-border partnerships and discuss good practice for local patient support groups. SCD patients were identified through ERN-EuroBloodNet SCD experts' members, National SCD Networks, ASCAT members and EHA ERN-EuroBloodNet representatives. Patients were then asked to brainstorm what issues relating to their care they felt requires research focus. These ideas were then grouped into themes, and formulated into individual research questions. Patients then voted on the questions of greatest importance to them, generating an overall "Top 10" research questions chosen by the patient community.

The brainstorming and theme grouping led to the formulation of 42 individual research questions. Patients were then given a hypothetical "10 million dollars" to spend on their top 10 projects, with the total spent on each project once all 28 participants had voted yielding an overall "Top 10" list representing what people living with sickle cell disease would like the research community to focus on.

Results

The workshop group included 28 participants coming from 7 EU countries, 5 non-EU countries. The group was made up of patients living with SCD, relatives looking after children with SCD and representing at least 10 National patient organizations. Geographical and gender balance has been taken into consideration. The workshop allowed patients to experience Patient and Public Involvement and to develop a meaningful list of questions which they would like research efforts to address.

In what we believe is a unique output, the patients presented the workshop outcome to the plenary session of the ASCAT congress, demonstrating that healthcare professionals are willing to change and listen to the patient voice. Future challenges will be to ensure that research funding is indeed channeled towards projects that address the issues identified by patients.

This following list represents what people living with sickle cell disease would like the research community to focus on:

1. How can we find a universally available cure for sickle cell disease?
2. How can we improve quality of life for people living with sickle cell disease?
3. What are the implications of sickle cell disease for work and education? How do we advise people, employers and schools?
4. How do we manage acute pain to prevent hospitalisation?
5. How does staff diversity or lack of diversity affect the way patients experience racism, discrimination and stigma?
6. How do we harness the political agendas to improve care for sickle cell disease?
7. How can we optimize pregnancy for mother and baby in sickle cell disease?
8. What complementary therapies work in sickle cell disease, including CBD oil and cannabis?
9. How do we manage chronic pain?
10. What is the impact of a comprehensive care team on outcomes for people living with sickle cell disease?

Priorities are available at the "Annex III ASCAT Poster Results"

At the end of the workshop a satisfaction questionnaire was submitted to the patients and the result of this questionnaire indicates a positive experience in unison. Questionnaire results in the "Annex IV ASCAT questionnaire results"

Next steps

- 1) Together with ASCAT members, ERN-EuroBloodNet will organize another SCD Patients Educational Session for ASCAT 2020.
- 2) ERN-EuroBloodNet will organize two patients session for the 4th Global Congress on Sickle Cell Disease (April 2020 date postponed due to Coronavirus pandemic, final date to be confirmed). This congress will bring together medical experts and researchers experienced in Sickle Cell Disease from around the world. During the congress, two sessions are dedicated to patient testimony; they will last 2 hours each. There will be 4 presentations of patient testimony of 20 minutes each + 10 minutes of audience questions.

4. CONCLUSIONS

One of the main objectives of the ERN-EuroBloodNet is to disseminate cutting-edge knowledge and facilitate continuous medical education (CME) in the field of RHD. For this aim, collaborations with educational bodies as EHA and ESH are essential, not only for the gathering of gaps of educational needs, but also for its analysis and plan which actions to address in order to close gaps.

The educational actions undertaken by the network during this period have allowed to address the major gaps identified during the previous period of network implementation by the establishment of a blended educational program for health professionals compounded by onsite and online actions addressing not only transversal topics, but also focusing on very concrete areas identified as not well addressed by the educational community so far, naming:

RHD transversal CME actions:

- ERN-EuroBloodNet Thursdays webinars for transversal knowledge on very rare diseases, complex disorders, highly specialized procedures and implementation of guidelines

Comprehensive programs on specific disorders:

- Webinars program on Cutaneous Lymphoma for health professionals to disseminate very innovative topics among health professionals in order to give visibility to the last cutting-edge advances in the field
- ERN-EuroBloodNet Preceptorships on Paroxysmal nocturnal hemoglobinuria and Aplastic Anemia as onsite program based on short stays on ERN-EuroBloodNet centers of expertise as the first area identified to be benefited from the organization of short stays due to the lack of expertise in its diagnosis and/or clinical management.

In addition, three major further actions have been addressed to enhance the education of patients through

- Definition of Webinars program on Cutaneous Lymphoma for patients' advocates and patients association understand diagnosis, treatments and management of specific type of Cutaneous Lymphoma to be better prepared to support within his/her patients' organization newly and already diagnosed patients. Starting in May 2020.
- The national meetings organized for the establishment of the European Network of Sickle Cell Disease Patients organizations that allow the introduction of patients in the "advocacy" meaning of the word while initiating them into the main steps to be part of the ePAG and ERNs community.
- Sickle Cell Disease Research Prioritisation Workshop at ASCAT that trained patients to understand their role on research

Moreover, the organization of Sickle Cell Disease Research Prioritisation Workshop at ASCAT, provided with the top 10 priorities to be most urgently tackled, allowing the identification of direct and indirect educational actions as:

- What are the implications of sickle cell disease for work and education? How do we advise people, employers and schools?
- How do we manage chronic pain?

In conclusion, ERN-EuroBloodNet educational strategy has allowed not only to directly promote the education of health professionals and patients, but also to initiate patients in the advocacy environment, while allowed the identification of priorities to be tackled with educational actions from the patients point of view.

Taking into consideration the wonderful feedback experienced from the initiatives, as general next steps the coverage of the actions will be expanded to other RHD areas already identified as urgent targets to be tackled, as preceptorship on sickle cell disease, or the expansion of the comprehensive webinars program to Haemochromatosis or other iron disorders.



European Reference Networks

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

Co-funded by the European Union

