Deliverable 5.1

Report on educational gaps

European Reference Network
for rare or low prevalence complex diseases
Network Hematological Diseases (ERN EuroBloodNet)

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Contents

1- Introduction

1.1 Rationale and background

One of the major challenges for rare diseases (RDs) is the promotion of the continuing medical education (CME) for both, professionals and patients, specifically addressed to these conditions. Given the low prevalence of these disorders, their presence at congresses and training, or the production of specific teaching and educational material has been traditionally under represented and are in many occasions difficult to find.

In the specific case of Rare Hematological Disorders (RHD), quality and specialized training courses focused on some RHD, especially those for non-oncological RHD and/or ultra-rare conditions, are infrequent and expensive since: a) expertise is scarce and those courses usually involve a high number of international speakers b) they include educational tools, such as webcasting for access on-line after the course and/or televoting educational exercises c) they address a small community.

In addition, curricula for medical specialty training differ from one Member State (MS) to another and, in general, little time is dedicated to ultra-rare conditions, where an educational action is urgently needed.

These educational gaps directly contribute to the low implementation of good practices in the field, leading to a variable and sometimes unequal management of patients affected by RHDs between countries.

Moreover, many unmet needs exist for educational material for patients. While more prevalent RHDs have well recognized disease specific educational material for the patients' community, the public information available for very rare RHDs targeting patients is very scarce, contributing in this way to increase the anxiety of affected population and their families who do not know where to search for reliable information on their diseases.

In this context, ERN-EuroBloodNet specific objective 3 aims to implement a blended educational program (on-site & on-line) with innovative contents and directed by the most outstanding experts in the different types of malignant and non-malignant RHD. Accordingly, the Transversal Field of Action (TFA) on Continuing Medical Education (CME) was established aiming to implement all tasks and activities related to the achievement of this objective, coordinated by Dominique Bron, as oncological representative, Patricia Aguilar Martinez as non oncological representative and Jan Geissler as ePAG representative for the Lymphoid disorders.

In this line, a collaboration among ERN-EuroBloodNet and two European well-recognized educational bodies, European Hematology Association (EHA) and European School of

Hematology (ESH), was already established in the first year of the network in order to joint efforts towards the better identification of educational gaps and needs on RHD as the basis for the establishment of a work plan to address them.

1.2 Objective and structure

This deliverable describes all the ERN-EuroBloodNet actions implemented during this period for the identification of educational gaps and its tackling in collaboration with EHA and ESH. The different actions reported includes the following sections:

- Rationale
- Objective
- Methods
- Results
- Next steps

2- Educational actions implemented

2.1 Repository of education materials available for Rare Hematological Diseases

Rationale

During the first year of ERN-EuroBloodNet implementation, EHA proposed three different approaches for the identification of educational gaps - Deliverable 5 "Report on actions foreseen by educational bodies (EHA and ESH) to address gaps identified in annual educational programme".

Based on the results of this analysis, it was agreed to conduct a two-step complementary surveys in collaboration with EHA comprised by:

- Part 1 – Questionnaire among ERN-EuroBloodNet members to create a repository of educational material and identification of educational needs.
- Part 2 – Questionnaire conducted among the EHA community to complement broader professional community and complement the results from the EuroBloodNet survey.

This section reports the design and results obtained from the conduction of Part 1. Part 2 of the questionnaire is currently being discussed among EHA, ESH and ERN-EuroBloodNet team.

Objective

To create a comprehensive repository of education materials available for Rare Hematological Diseases (for both health professionals and patients) to:

- Make available material that may not be known to other professionals or patients.
- Identify which are the educational needs within the network in order to be addressed in the coming annual work plan with the cooperation of EHA and ESH.

Methods

ERN-EuroBloodNet questionnaire on Continuing Medical Education

For the conduction of the questionnaire, TFA on CME coordinators and ERN-EuroBloodNet coordination team prepared an Excel including two tasks:

Task 1 : List of educational materials available by disease group

An excel sheet was created for each subnetwork for the collection of educational material, including sections to:

- Give the title of the educational material
- Indicate if A- Teaching material or B- Patient Education

- Indicate if A- Available in your centre or B - External material
- Language/s of the material available
- Link or indicate annex (PDF, PPT)
- Select group of diseases covered by the material among the disease-categories defined for each subnetwork

Task 2: Assessment of the educational needs

Four questions were defined to assess educational needs by providing the possibility to express two single binary answers. First concerns the affirmation or denial of a need (yes/no). Second underlines if the need is correlated to CME or therapeutic patient education (patient specific/health professional specific). Three free-text boxes were provided to specify the disease/ the group of diseases, to describe the educational need and why it is required.

The four questions that compile the task 2 are:

- Q1. Do you have specific educational needs in your field of expertise?
- Q2. Do you have specific educational needs out of your field of expertise?
- Q3. What would be – according to your opinion - the unmet educational needs that should be filled by the ERN, in order of priority? (e.g. Teaching material, guidelines, patients' education… Please list a maximum of 5.)
- Q4. Additional comments

In order to get the maximum number of answers from the experts on RHD, especially for those ultra-rare RHD, ERN-EuroBloodNet coordination team sent via email to members two rounds of the questionnaire. Also during the scientific and strategic board and Board of the network meetings it was encouraged the activity of answering to the survey.

Results

All the surveys received where collected in a single excel file. Answers to binary and close questions were quantified and categorized.

A total of 21 answers compiling feedback from 27 experts were received from ERN-EuroBloodNet members belonging to 9 European Member States (Fig 1). List of contributors is available in **Annex I List of ERN-EuroBloodNet experts responders to the Questionnaire on CME**.

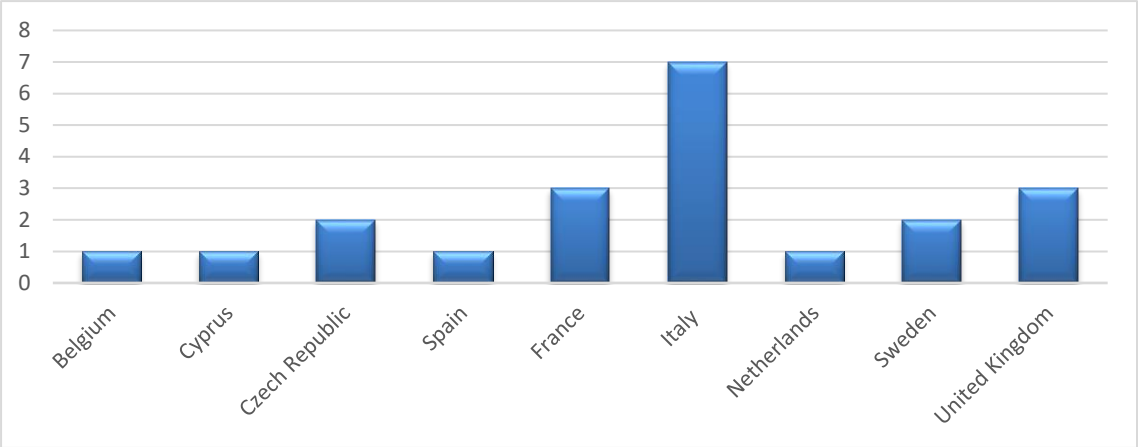

Figure 1. Number of answers received for the ERN-EuroBloodNet questionnaire on CME by country.

Task 1 : List of educational materials available by disease group

A total of 152 educational materials were compiled and classified according the criteria assigned in the template. Material compiled by subnetwork is available in **Annex II List of educational material.** The coverage of the material compiled by subnetwork is illustrated in figure 2.

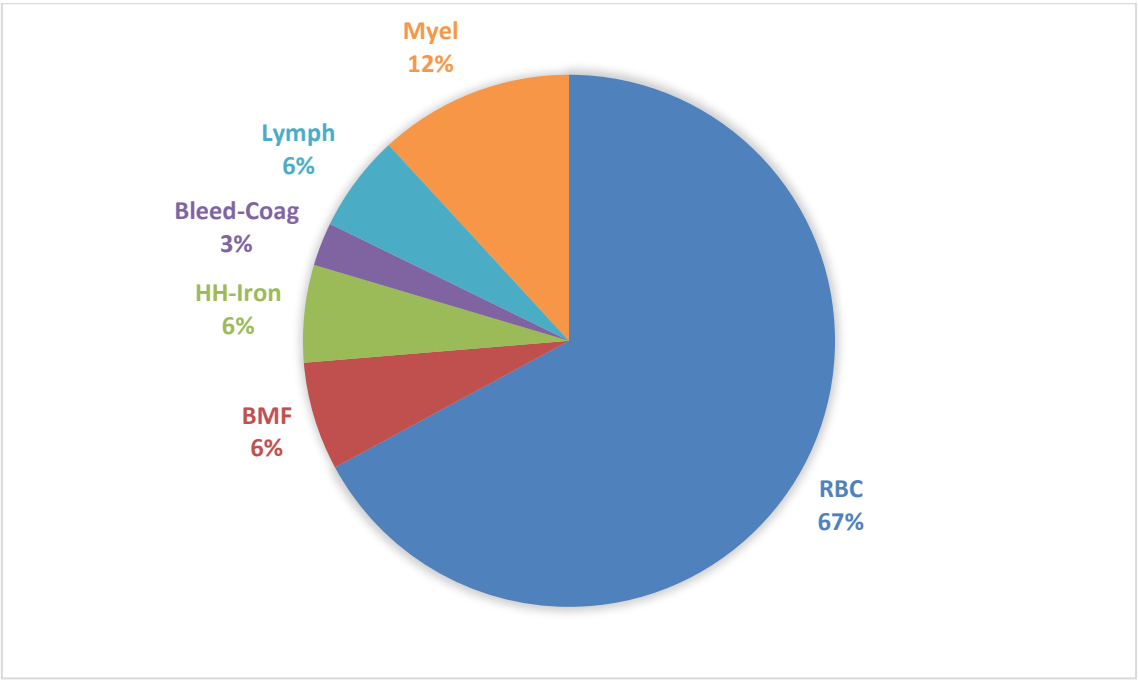

Figure 2. Coverage of the Educational material compiled through the ERN-EuroBloodNet questionnaire on CME by subnetwork (RBC: red blood cell defects, BMF: Bone marrow failure and hematopoietic disorders, HH-Iron: Hemochromatosis and other rare genetic disorders of iron metabolism and heme synthesis, Bleed-Coag: Rare bleeding-coagulation disorders and related diseases, Lymph: Lymphoid malignancies, Myel: Myeloid malignancies).

Regarding the target (health professionals, patients or both) and accessibility (available in the center, external or both) of the educational material compiled, Table 1 and Figures 3 and 4 show the classification of the material by subnetwork.

	Teaching material	Patient education	Both	Total	Available in center	External material	Both	Total
RBC	60	31	11	102	37	30	35	102
BMF	9	0	1	10	9	1	0	10
HH-Iron	5	1	3	9	5	4	0	9
Bleed	2	1	1	4	0	4	0	4
Lymph	6	3	0	9	7	2	0	9
Myel	8	10	0	18	7	10	1	18

Table 1. Distribution of educational material regarding targets and accessibility

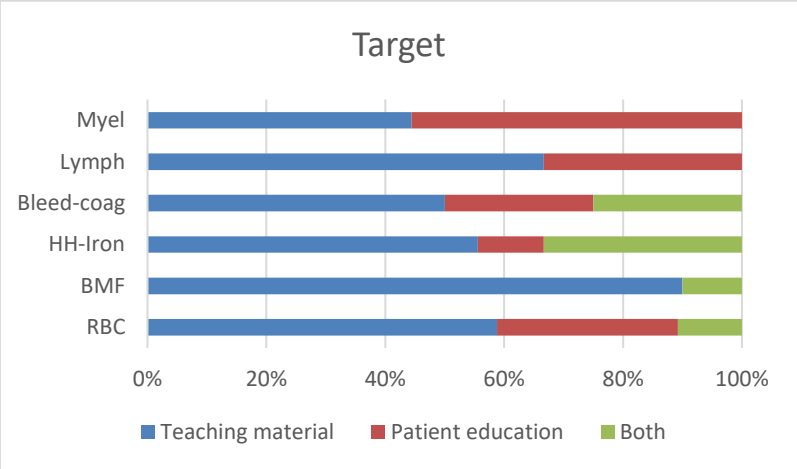

Figure 3. Distribution in % of educational material regarding targets by subnetwork

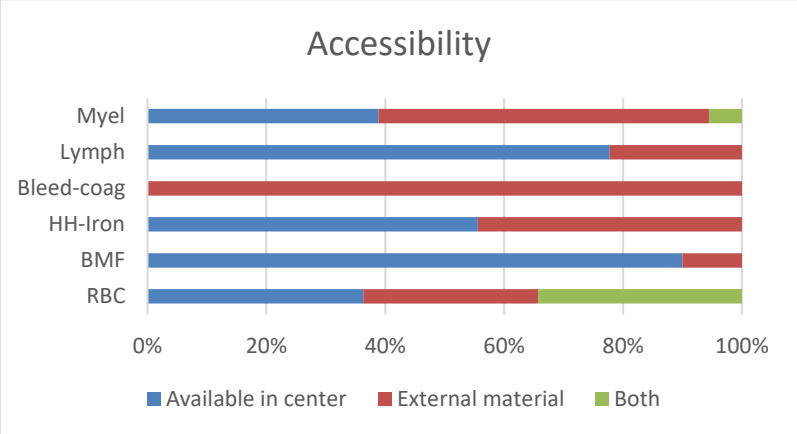

Figure 4. Distribution in % of educational material regarding accessibility by subnetwork

Material compiled for the different diseases subgroups included under each subnetwork was also analysed. A significant number of material covers more than one of the subgroups or diseases contemplated for the classification. Attending to the type of the material compiled for the different subgroups of disorders, significant differences are contemplated across the subnetworks as shown in the figures below:

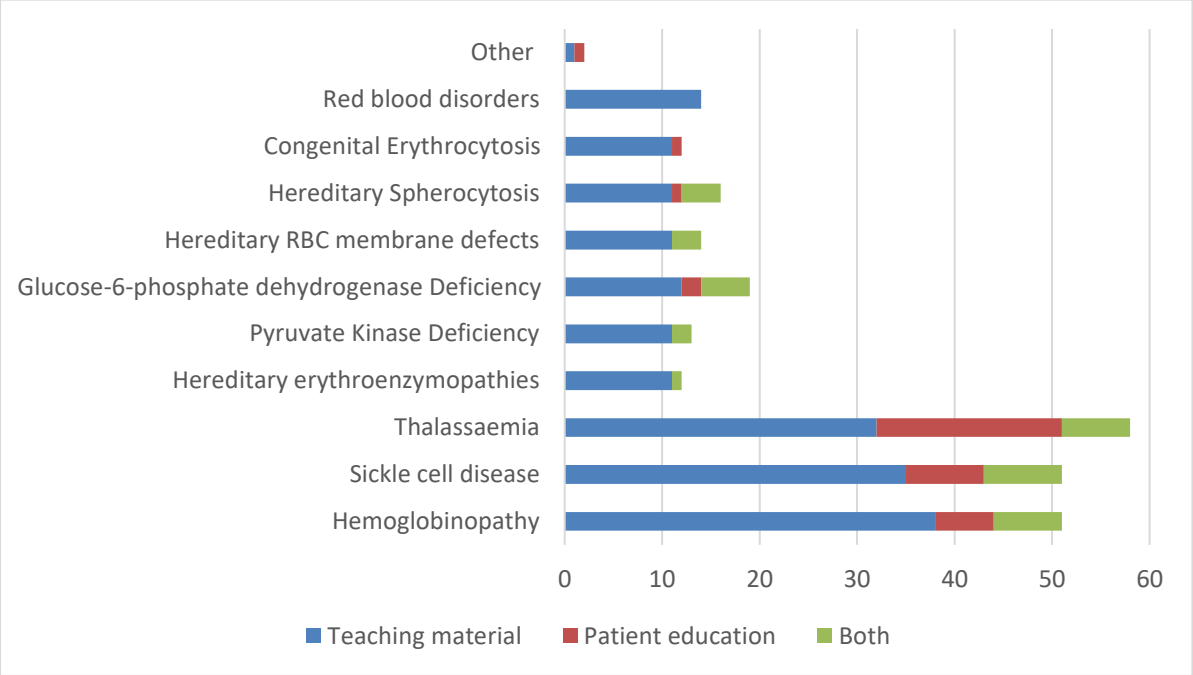

Figure 5. Material compiled for the RBC diseases categories

For the RBC subnetwork there is a clear dominancy of the material compiled addressing the haemoglobinopathies, category that includes Sickle cell disease and thalassaemia disorders. In addition material is especially targeted to health professionals. This result is logic since a) haemoglobinopathies are the most prevalent disorders in EU within the red blood cell subnetwork, b) Thalassaemia is endemic on the Mediterranean area (Italy, Cyprus), from where many answers were received and c) sickle cell disorders is endemic in Africa, and countries receiving especial migrant flows from there have provided many material targeting these disorders (United Kingdom, France). Moreover there is a high number of materials for patients on the Thalassaemia area, which could be explained for the well-established Thalassaemia International Federation (TIF), that counts with decades of background supporting the

thalassemic patients community in different areas of action, being one of them the generation of educational material for patients.

On the other hand, it is remarkable the low material available for patients focused on erythroenzymopathies (including Pyruvate Kinase Deficiency and Glucose-6-phosphate dehydrogenase Deficiency), RBC membrane defects (including spherocytosis) and erythrocytosis, where for some of them there is a lack of material only addressed to patients. Accordingly, although covered by some material for both areas, still more efforts are needed for the generation of patients' oriented material.

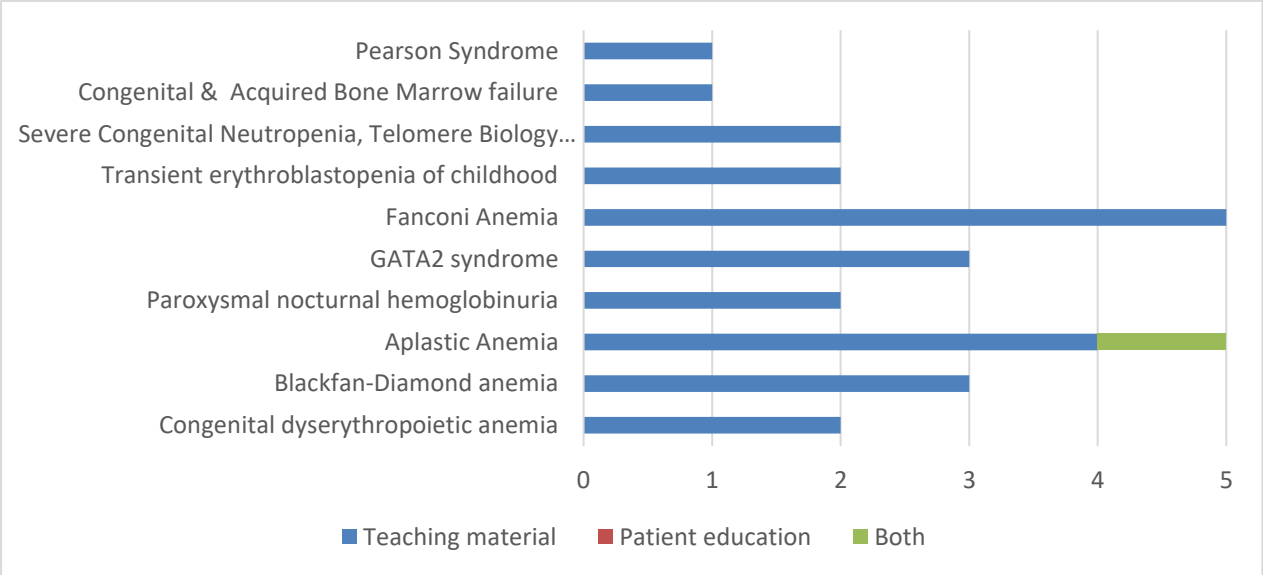

Figure 6. Material compiled for the BMF diseases categories

For the Bone marrow failures subgroups, it is remarkable almost the total lack of material addressed to patients, only contemplated by one material covering both, teaching and patients' education for Aplastic Anaemia. There is a clear need of promotion for the production of more material targeted to patients affected by these disorders.

On the other hand, there are one disease especially poorly covered by education material, Pearson Syndrome. In addition, if looking at the accessibility of this material, it is only available by the center provided, accordingly, these diseases could be a well gap to be addressed by the generation of new material.

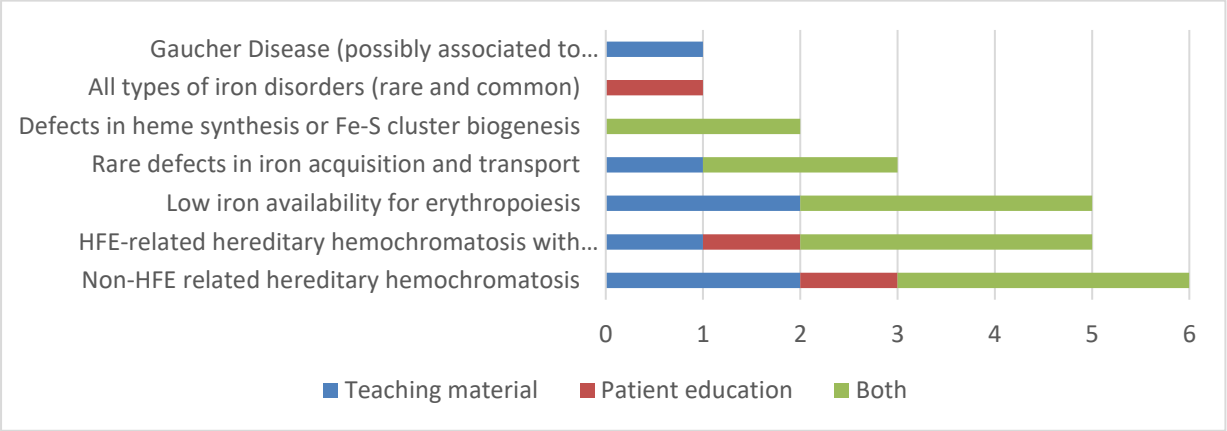

Figure 7. Material compiled for the HH-iron diseases categories

For the HH-iron subnetwork it is highlighted the high number of material available for patients, or in especial for both, health professionals and patients. This is a reflect of the deep involvement that this group of patients have in the community, relation also demonstrated with their high grade of involvement in the development of guidelines in the field (Deliverable 4.1 Repository of guidelienes).

A potential gap to be covered is material for patients' education on the Gaucher Disease, which is currently lacking in the repository so far.

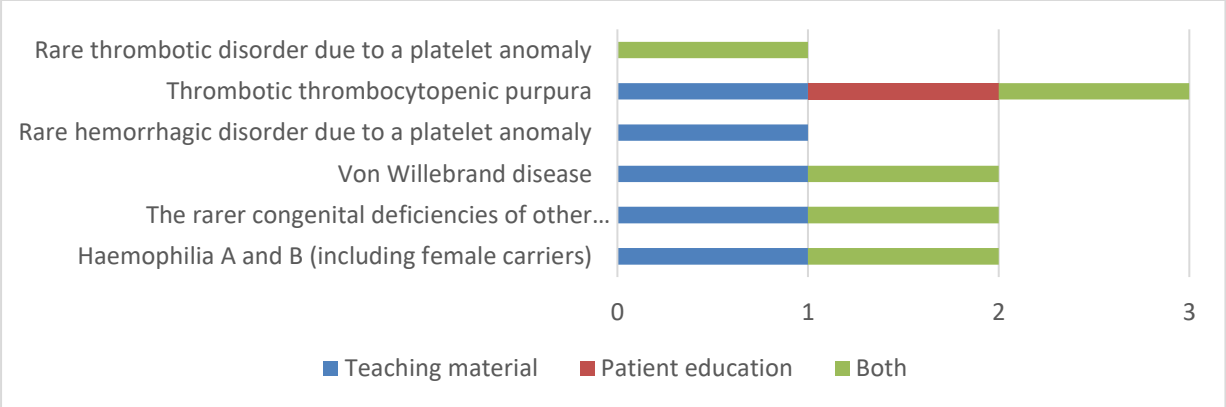

Figure 8. Material compiled for the Bleed-Coag diseases categories

The bleeding-coagulation disease categories are well balanced by the coverage of material for patients and professionals, with the exception of the Rare hemorrhagic disorders due to a platelet anomaly, for which material for patients have not been compiled.

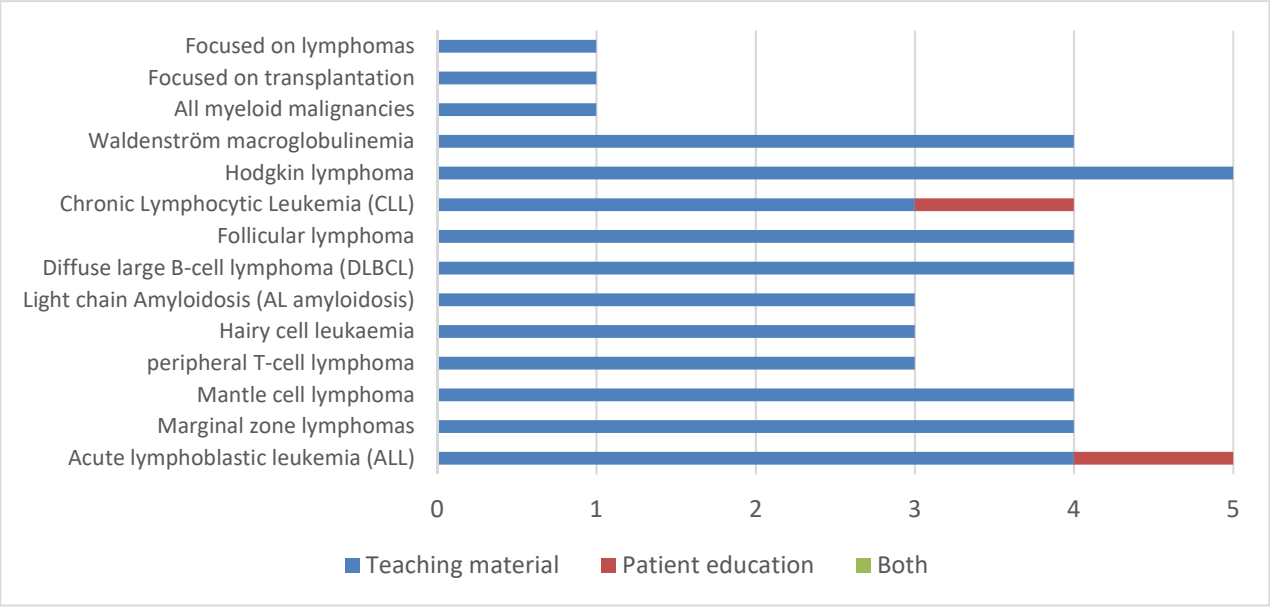

Figure 9. Material compiled for the Lymphoid malignancies categories

For the lymphoid malignancies, as seen for the bone marrow failures, there is an evident need of generating material for patients' education. With the exception of ALL and CLL, all the entities could be target for addressing this gap.

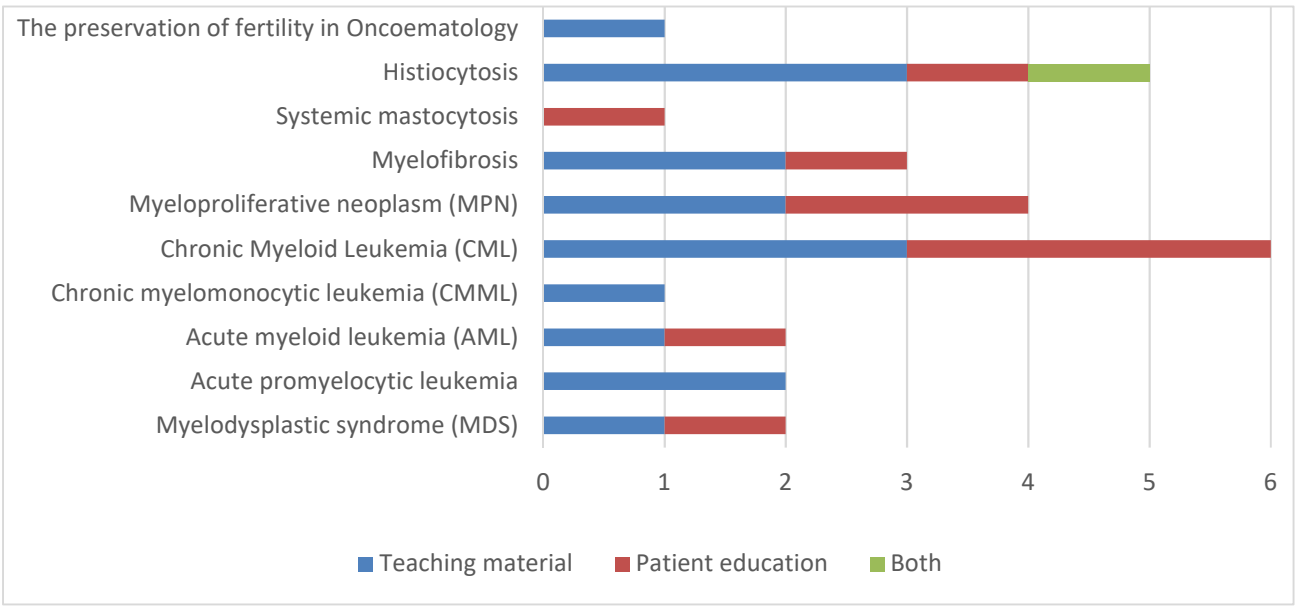

Figure 10. Material compiled for the Myeloid malignancies categories

For the Myeloid subnetwork, there is a quite balance on the coverage of the material found for both patients and professionals with two exceptions that could be targeted in the coming steps of the network: CMML and Acute promyelocytic leukemia.

Task 2: Assessment of the educational needs

Q1 Do you have specific educational needs in your field of expertise?

A total of 34 disease focused educational needs were gathered, 15 of them to be addressed to health professionals and 16 for patients. In 3 of them the target was not specified. Their distribution by subnetworks are shown in Figure 11.

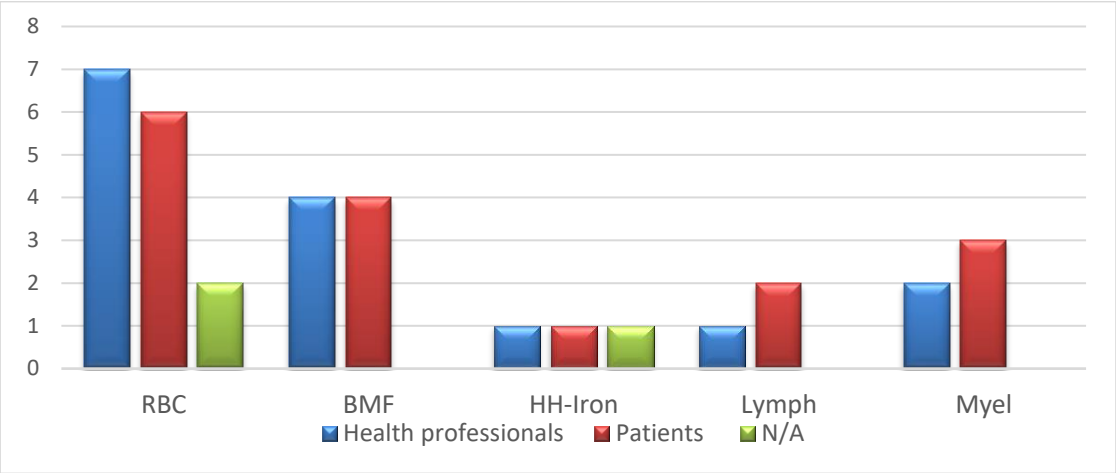

Figure 11. Number of needs by subnetwork and target.

In the area of RBC for health professionals, it has been required teaching material for Haemoglobinopathies specifically focused on Next Generation Sequencing based diagnosis and Proteomics, and teaching material for the diagnosis and clinical management of Enzymopathies, Membranopathies and Other Hemoglobinopathies (except Thal and SCD) given the lack in some centers of national recommendations. The main outcome for patient is to have educational material in those RBC conditions above mentioned. In addition, it has been required material for SCD, like visual to work out: posters, booklet for children and adults in creole, video support starting from a part of the booklet, genealogic trees. This request has been formulate because SCD patients come from different cultures and educational levels. Finally, it has been underlined that all hemoglobinophaties would require a multidisciplinary approach with the involvement of family doctors that should disseminate the knowledge on therapeutic options and treatment of chronic complication and genetic counseling.

For the BMF subnetwork, one of the most relevant result is the request of therapeutic educational material for patients and the request of teaching material for the diagnosis and

clinic management of Diskeratosis congenital, Blackfan Diamond Anemia, Amegacaryocytic thrombocytopenia, given the lack of national guidelines.

Attending to the HH-Iron subnet, outcomes showed a common request among experts' centers of having available material both for patients and health professionals concerning the diagnosis and clinical management of heme group diseases. A specific request has come asking for available material regarding the iron chelating drugs in MDS and "atypical microcytic anemias" with iron overload, because for iron chelating drugs in non-typical indications is mainly followed the expert-opinion.

Analyzing the lymphoid subnetwork answers, for health professional it has been required teaching material for: plasmoblastic lymphoma, granuloma, diffuse large B-cell lymphomas, HS lymphomas. For patients it has been expressed the need to have therapeutic patient education material for lymphoid malignancies, i.e. the material is scarce and provided by pharmaceutical societies.

Lastly, for the myeloid subnetwork, two relevant results have been obtained, one concerns the need of having valuable material for MDS in genetic risk, assessment and diagnostics. The other concerns the need in patient education of material for myeloid malignancies.

Q2. Do you have specific educational needs out of your field of expertise?

In this question, experts were asked about the educational needs out of their expertise from their point of view. A total of 11 answers were received affirming the need and 17 denying. A total of 7 educational needs were identified for the health professional area and 4 for patients. Their distribution by subnetworks are shown in Figure 12

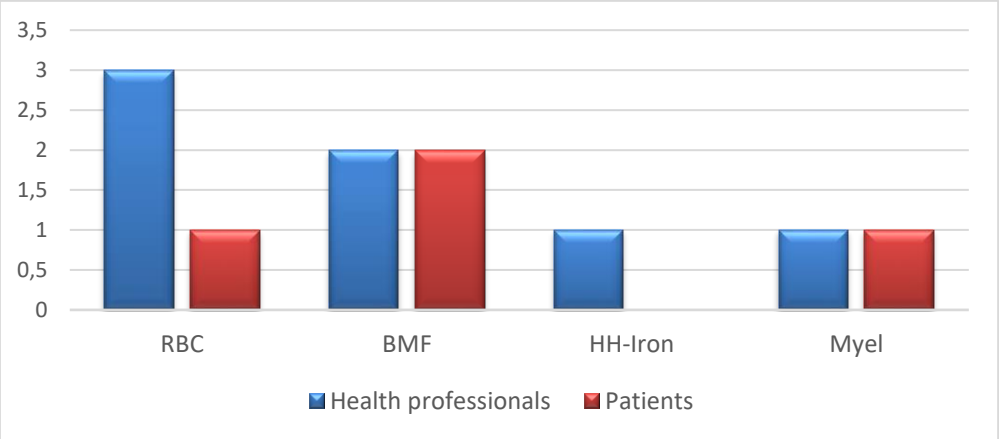

Figure 12. Educational needs identified for health professionals and patients by subnetwork.

For the RBC the result that stands out more is the requirement of having educational material in the diagnosis of the rare forms because they could be uncommon at the national level. For instance a center has expressed the need to have for Haemolytic rare Anaemias other than Haemoglobinopathies, diseases courses, seminars, guidelines for carrier screening in order to launch a process of implementing in routine clinical practice.

On the other hand, for BMF one of the outcome is the need of having novel pan-European guidelines and the need of more valuable material on genetic diagnostic. Also for the patient it has been underline the difficulties to know how to access better diagnostic and therapeutic care.

In the HH-iron subnetwork, a center raised the importance of having material for paediatric age.

For myeloid disorders, the most interesting outcome is the need of having European guidelines in myeloid malignancies with germline background and material for the patient awareness for knowing how to access better diagnostic and therapeutic care.

Q3. What would be – according to your opinion - the unmet educational needs that should be filled by the ERN, in order of priority? (eg. Teaching material, guidelines, patients education… Please list a maximum of 5.)

31 answers received, being 3 of them n/a and 1 was general for RHDs. Number of answers compiled by subnetwork are summarized in Figure 13

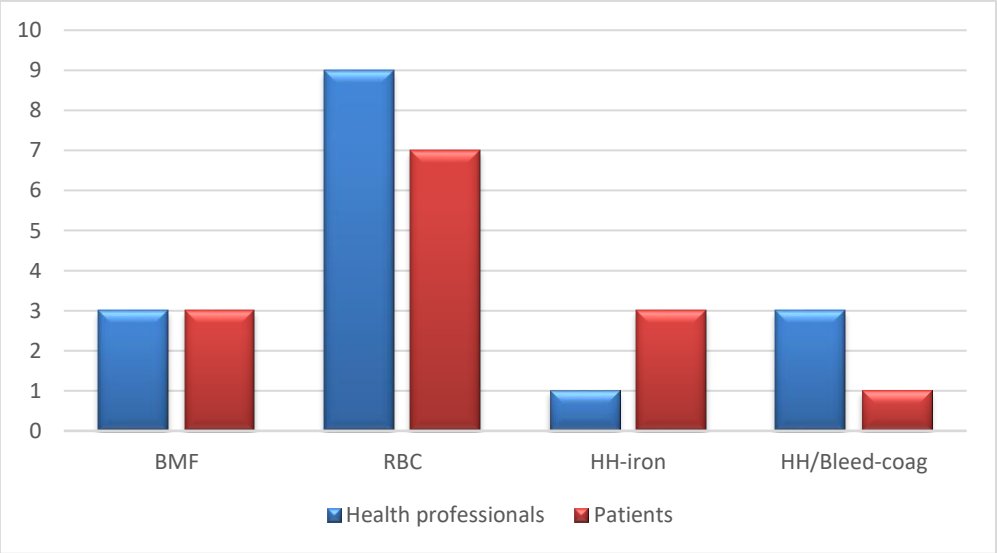

Figure 13. Number of answers to Q3 by subnetwork.

For RBC one of the outcomes was the establishment of guidelines in Haemolytic rare Anaemias, RBC disorders, except thalassemia and SCD, because they are not always available at national level. In SCD and Thalassemia it has been raised the need of having therapeutical patient education material and common European recommendations and teaching materials with simulation of cases because of the clinical management of both chronic and acute complications.

For the BMF subnetwork, the need of guidelines and teaching material both for health professionals and patients was highlighted given the lack of available recommendations.

In the HH-Iron subnet one of the outcomes obtained is the need of guidelines and teaching material in Iron chelating drugs in MDS and "atypical microcytic anaemias" with iron overload because the current usage of iron chelator in non-typical iron overload is mainly based on expert-opinion.

A comment on both, HH-Iron and Bleed-Coag subnet was for considering the great variability in clinical, laboratory and instrumental presentation of disorders of iron metabolism and heme synthesis and rare bleeding-coagulation disorders. It has been raised the need of establishing a repository of clinical cases (clinical, laboratory, instrumental findings ; II and III level diagnostic work-up ; diagnosis ; treatment). For the same reason it has been required the organisation of educational activity with case-based scenarios.

The harmonization of the therapeutic patient education materials gathered by the ERN EuroBloodNet in a multi-lingual repository has been also identified by many experts as transversal RHD request.

Q4. Additional comments

The importance of having the educational material in local language was highlighted in order to facilitate their dissemination among patients and health professional.

The ERN-EuroBloodNet is asked also to be sure that guidelines are updated and get into use by all hematologists and get disseminated among patients.

2.2 Repository of Sickle Cell Disease Therapeutic Patient Educational material

Rationale

Sickle cell disease (SCD) is an inherited disorder of the red blood cells. It is a lifelong chronic condition. If it affects the patient in a severe form it can lead to disability or even premature death.

Up to now, SCD is one of the few rare anemias that does not have, in every country in Europe, an adequate educational contribution to 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 therapeutic educational needs of the SCD patients is 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, and they could face, in most cases, many integration difficulties of a population largely coming from third countries, as for instance to have access to cares and to assert social rights.

Accordingly, ERN-EuroBloodNet is currently identifying the Sickle Cell Disease patients' needs across European Member States in order to better analyze the existing gaps and try to face them through dedicated actions. After an analysis undertaken by the ERN-EuroBloodNet coordination team, Angelo Loris Brunetta (ePAG RBC subnetworks coordinator), Dr Raffaella Colombatti (expert member of SCD) and TFA coordinators of CME, the gathering of the educational material for SCD patients available within the network and among patients organizations across EU MS was identified as an urgent need.

Objective

To create a European repository of Sickle Cell Disease educational material in order to

- Make those materials accessible for every patient
- Identify educational gaps

Methods

The SCD educational material for patients was compiled from:

a) EuroBloodNet healthcare professionals: from Part 1 of the ERN-EuroBloodNet Questionnaire on continuing medical education (CME).

b) SCD patients' organizations across EU:

A parallel Questionnaire on the available educational material for SCD patients was defined with the objectives to:

- Expand the educational material available compiled via the questionnaire on CME
- Collect patients' opinion about the therapeutic patient educational domains to be covered.

Questionnaire on the available educational material for SCD patients

The survey was designed by ERN-EuroBloodNet coordination team and Angelo Loris Brunetta (ePAG RBC subnetwork coordinator), and prepared in an excel including two tasks:

Task 1: To gather the educational material used/known for SCD.

It gives the possibility of a free-text answer for each Excel box:

- Title of the educational material
- Indicate if A- Available in your patient organization or B - External material
- Indicate if cover: Adult/Pediatric/Transition
- Format (training course, e-learning plateforme, book, article, video, mobile application, interactive game, etc)
- Language/s of the material available
- Link or indicate annex (PDF, PPT)

Task 2: To gather educational needs from the patients' perspective.

1. First free-box question: which is your country of residence?
2. Second question: do you have any specific educational needs?
3. Third question: what would be – according to your opinion - the unmet educational needs that should be filled by the ERN, in order of priority? Please list a maximum of 5.

For the second question is possible to give affirmative answers by a tick in the appropriate box, and add any comments. The educational needs are expressed and classified by the following categories:

a) Educational need linked to patient adaptation process:
   - Management of health (understanding the state of the health just after diseases discover, understandings of the medical conditions, diagnoses, disease)

b) Educational need linked to patient compliance:
   - Management of the daily life

- Management of pain crises and factors that interfere with the normal management of disease conditions
- Management of the treatment (i.e. how to take it, recognition of side effects , understandings of risk of not regularly take it or mixing other substances, recognition of situations that require medical advice or a physician visit)
- Management of psychological well-being

c) Educational need linked to patient experience:
- Know how to advocate/being part of a patient organisations
- Know how to use medical services (i.e. scheduling visits, using social services, etc)
- Knowledge of RDs policy in health system (rights for patients, economical supports, etc.)
- Be aware of cutting-edge research on SCD
- Know how to read a scientific article
- Know how to be involved in research

d) Educational need linked to patient surrounding people:
- Acknowled of SCD conditions by family, friends, school teachers, homecare facilitators, employers and collegues

e) Other

In addition, a line is crossed with educational need column. It is the items to express the reasons of the educational need and to which patient age the need belongs to.

- Pediatric/Adult/Transition between pediatric and Adult
- Lack of training centers/teachers
- Lack of educational training online (e-learning programs, webinars)
- Lack of digital tool and numerique material (i.e. mobile apps, online informations,etc.)
- Lack of printed material
- Cannot access to material
- Lack of information about how to access to education offering
- Lack of free educational offering
- Lack of patients organisation nearby
- Other reasons/ comments

Next step

- The survey will be translated in French, Spanish and Italian in order to reach a good number of patients' organizations. Sickle Cell Diseases patients in Europe speak mostly English and French.
- A list of SCD patients' organizations existing among EU MS has been established. It has been created consulting ENERCA patients associations search tool, Orphanet patients associations search tool, Google search. SCD EuroBloodNet experts' members and French network of RBC (Filière MCGRE) has implemented the list with their data. Those associations listed will be contacted by email by the ERN EuroBloodNet team with an invitation to answer to the survey. Invitation letter will be also translated into English, Spanish, French and Italian.
- The survey will be conducted during 2019. A report will be prepared with main results.
- A repository with sharable material will be created and made available on the EuroBloodNet webpage.
- Therapeutic patient education needs expressed both by experts and by patients organizations will be addressed in the coming annual work plans in order to consider how to promote these needs and close the identified gaps.

2.3 Webinars for health professionals

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 nourish from the most outstanding experts in the field.

In this context, both, webinars and presentations with audio for health professionals have been included in the multi annual-educational programme promoted by ERN EuroBloodNet.

Objective

The aim of these online educational activities is to contribute to continuing medical development requirements of health professionals, assisting them to provide cutting-edge knowledge on very rare diseases and highly specialised procedures, avoiding the general speech

around a rare disease, but focussing on a very innovative and specific disease, clinic or intervention area.

Methods

Webinars description

Webinars will be conducted using the Webex Platform provided by the European Commission. It allows full audio and visual communication, as well as interaction between webinar's speaker and participants.

Webinars will be led by the speaker with the visual aid of a Power Point presentation shown through the "shared screen" option of the Webex Platform. Webinars sessions will be finally recorded using the Webex record tools. The mp4 audio got by the session and the power point presentation will be uploaded and made available on the ERN-EuroBloodNet webpage.

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 (foreseen Thursdays at 17:00).

ERN-EuroBloodNet will implement 1-2 webinars per month. The balance among subnetworks will be ensured, holding 2-4 webinars/year/subnetwork.

List of potential webinars

In order to organize high-impact webinars, topic focussed sessions will be preferably focused on very rare disease, highly specialised procedures and very innovative areas where education needs to be promoted. In addition, each webinar will be tailored on a target group of health professionals.

In order to list potential target topics to be covered by the webinars, ERN-EuroBloodNet, within the TFA CME task force has analysed the results from the survey "ERN-EuroBloodNet questionnaire on CME" and the List of international guidelines and recommendations – "Deliverable 4.1 Report on the comprehensive public database of reliable guidelines".

Results

List of potential webinars

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. As a result, the following list of potential webinars and speakers have been created:

1. Webinar on the Recommendation: "Addressing the diagnostic gaps in pyruvate kinase deficiency: Consensus recommendations on the diagnosis of pyruvate kinase deficiency" - Paola Bianchi, HCP Foundation IRCCS Ca'Granda Ospedale Maggiore Policlinico, Milan.

The webinar would increase the awareness of the recommendations recently published under the endorsement of ERN-EuroBloodNet, providing other centres the possibility to deliver timely and appropriate diagnosis of pyruvate kinase deficiency.

2. Webinar on "Newborn screening for sickle cell disease in Europe: recommendations from a Pan-European Consensus Conference" has just been published with the endorsement of ERN-EuroBloodNet - Raffaella Colombatti, HCP AO Padoua.

These recommendations produced from a Pan-European Consensus Conference have been recently published under the endorsement of the network. The early detection of the disease through Newborn screening (NBS) is a key factor to initiate preventive measures that allows improving the prognosis of the disease, and thus, leading to a reduction in childhood mortality rate due to SCD.

3. Bone marrow failure Subnetwork:

An important educational need has been identified in diagnosis and clinical management of:

- Dyskeratosis congenital. One session could be centred on "Diagnosis and management of congenital dyserythropoietic anemias" coming from the homonym cutting-edge publication of HCP AOU Federico II, Naples, cfr https://www.ncbi.nlm.nih.gov/pubmed/26653117.
- A second session linked to this disease could be focused on "Recommendations regarding spelenectomy in hereditary haemolytic anemias" cfr https://www.ncbi.nlm.nih.gov/pubmed/28550188 a study conducted by a high number of EuroBloodNet members.
- Blackfan Diamond. One session could be dedicated to "Anemia Diagnosing and treating Diamond Blackfan anaemia: results of an international clinical consensus conference" cfr https://www.ncbi.nlm.nih.gov/pubmed/18671700
  A second session could talk about "how I treat the Diamond-Blackfan anemia" based on this following guideline, cfr https://www.ncbi.nlm.nih.gov/pubmed/?term=How+I+treat+Diamond-Blackfan+anemia
- The possibility of organizing specific webinars on the subject of BMF genetic diagnostic will be analyzed

4. For what concerns HH-iron Subnetwork

It has been raised the lack of specific guidelines regarding diagnosis and clinical management of HH heme group diseases. A special attention should be given to two aspects: pediatric age; and current usage of iron chelator in non-typical iron overload.

- Hereditary hemochromatosis: A webinar session could be dedicated to these following guidelines on genetic testing: "EMQN best practice guidelines for the molecular genetic diagnosis of hereditary hemochromatosis (HH)", a study conducted also by two of EuroBloodNet members, cfr https://www.ncbi.nlm.nih.gov/pubmed/26153218 another topic coming from published guidelines could be "The quality of hereditary haemochromatosis guidelines: a comparative analysis", cfr https://www.ncbi.nlm.nih.gov/pubmed/25441394 One of the authors is an EuroBloodNet member coming from the HCP Radboud University Medical Center Nijmegen).
- Microcytic anemias: Another candidate topic is: "Practice guidelines for the diagnosis and management of microcytic anemias due to genetic disorders of iron metabolism or heme synthesis". Coming from the homonym publication where two HCPs (Radboud University Medical Center Nijmegen and University Medical Center Utrecht) have contributed. Cfr https://www.ncbi.nlm.nih.gov/pubmed/24665134

5. For what concerns RBC Subnetwork

It has been expressed the educational need, in diagnosis and clinical management of: enzymopathies, membranopathies and other hemoglobinopathies. It has also been raised the necessity to fullfill the gap regarding the next Generation Sequencing based diagnosis, and Proteomics for the Haemoglobinopathies.

- Enzymopathies a webinar could be focalized on this guidelines "Preterm Neonates: Beyond the Guidelines for Neonatal Hyperbilirubinemia" cfr https://www.ncbi.nlm.nih.gov/pubmed/27235216
- Membranopathies a webinar could be focalized on those guidelines: "Guidelines for the diagnosis and management of hereditary spherocytosis – 2011 update", one of our member experts, prof. Achille Iolascon, has participated to this study, cfr https://www.ncbi.nlm.nih.gov/pubmed/22055020

  Other topic candidate for a webinar session could be: "ICSH guidelines for the laboratory diagnosis of nonimmune hereditary red cell membrane disorders" coming from a

publication made with the contribution of some EuroBloodNet expert member cfr https://www.ncbi.nlm.nih.gov/pubmed/25790109

Finally another potential focus taken into consideration by a Webinar speaker could be "Preterm Neonates: Beyond the Guidelines for Neonatal Hyperbilirubinemia"

- Other Hemoglobinopathies (except Thal and SCD) "EMQN Best Practice Guidelines for molecular and haematology methods for carrier identification and prenatal diagnosis of the haemoglobinopathies" cfr https://www.ncbi.nlm.nih.gov/pubmed/25052315 in this work some experts members have participated.
  It could be also possible to focalise a specific session on "Significant haemoglobinopathies: guidelines for screening and diagnosis" cfr http://onlinelibrary.wiley.com/doi/10.1111/j.1365-2141.2009.08054.x/abstract
- Thalassemia A special session addressed to nurses continuing formation could follow this publication "A guide for haemoglobinopathy nurse" cfr http://thalassaemia.org.cy/publications/tif-publications/a-guide-for-the-haemoglobinopathy-nurse-2013/
- Polycythaemia/erythrocytosis: "Guidelines for the diagnosis, investigation and management of polycythaemia/erythrocytosis" cfr https://www.ncbi.nlm.nih.gov/pubmed/?term=Guidelines+for+the+diagnosis%2C+investigation+and+management+of+polycythaemia%2Ferythrocytosis
- It will be examined the possibility of organizing specific webinars on the subject of next Generation Sequencing based diagnosis, and Proteomics for the Haemoglobinopathies.

6. For what concerns Myeloid malignancies subnetwork

It has been raised the educational need for genetic risk assessment and diagnostic for MDS, and myeloid mal with germline background.

- A possible axes to cross with Webinar would be "Diagnosis and treatment of primary myelodysplastic syndromes in adults: recommendations from the European LeukemiaNet" from a cutting-edge publication to which some EuroBloodNet expert have participated, cfr https://www.ncbi.nlm.nih.gov/pubmed/23980065

7. For what concerns Lymphoid malignancies subnetwork

It has being raised the need of covering the healthcare domain of very rare lymphoproliferative disorders.

- Diffuse large B-cell lymphoma (DLBCL) A webinar session could be based on the results of "ESMO Guidelines consensus conference on malignant lymphoma 2011 part 1: diffuse large B-cell lymphoma (DLBCL), follicular lymphoma (FL) and chronic lymphocytic leukemia (CLL)" cfr https://www.ncbi.nlm.nih.gov/pubmed/23175624

Next steps

- The list of final topics will be agreed and potential speakers will be officially invited for the in order to finalize the annual webinars program.
- The program will be released among ERN-EuroBloodNet members and scientific societies in order to allow the interested participants to block the dates with months in advance.
- Webinars are expected to start by September 2019.

2.4 Preceptorships for health professionals carried out on HCPs sites and coordinated by ERN-EuroBloodNet experts

Rationale

In order to have an exhaustive Continuing Medical Education, some highly specialized procedures or diagnosis and clinical management of very rare diseases would require not only lessons but also practical exercises held in experts' centers. Based on the results of the EuroBloodNet Questionnaire on continuing medical education" and on the feedback from subnetwork coordinators, some areas for on-site training and experts centers related, have been identified.

During first year of ERN-EuroBloodNet implementation Paroxysmal nocturnal hemoglobinuria (PNH) 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.

Highly specialized centers that will host the preceptorships are chosen among EuroBloodNet Health Care Providers and represent the excellence in RHDs field at International level.

Objective

The priority aim of preceptorship is not simply to consider the global lack in cutting-edge education for physicians, but moreover to spread the expertise in countries where expertise on RHD is scarce.

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

Perceptorships aim to discuss AA, PNH, and other related bone marrow failure syndromes and to provide applicants with the fundamental tools for a correct diagnostic and treatment approach to marrow failures, including AA and PNH in children, adolescents and adult patients. 3 or 4 days program will be held in 3 highly specialized centers of the ERN EuroBloodNet. Each preceptorship will be attended by 4 participants.

Highly specialized centers that will host the preceptorships are:

1) AP-HP Hôpital Saint-Louis, Paris, France. Coordinator: prof. Régis Peffault de Latour.
2) University of Naples Federico II, Naples, Italy. Coordinator : prof. Antonio Risitano.
3) Pediatric Hospital - IRCCS Institute Giannina Gaslini, Genoa, Italy. Coordinator: prof. Carlo Dufour.

Results

Sessions will have several formats: formal lectures, informal discussion, case study discussion, and direct tutorial activity (in the clinic and/or in a lab). Full description for the Preceptorship in each center (Teachers, topics, description and agenda), are included in **Annex III AA and PNH Precerptoship program**

Next steps

- A call for participants will be established and candidates will be chosen by a jury according to curriculum vitae and cover.

2.5 EHA and ERN-EuroBloodNet ePAG Patient Advocacy Capacity Building Meeting

Rationale

The European Hematology Association celebrates each year its annual congress with the attendance of more than 11,000 attendees from all fields of hematology. Taking advantage of this perferct environment for, learning, training, discussion and brainstorming on hematological disorders, an agreement was reached during the first year of the ERN-EuroBloodNet implementation with EHA and ePAGs representatives to reinforce the sessions dedicated to patients within the EHA congress.

Objective

To reinforce the sessions dedicated to patients celebrated within the EHA congresses following ePAGs demand to not duplicate number of symposiums for patients but collaborate with the EHA for its strengthen during the biggest annual congress on Hematology at EU level.

Methods

ERN-EuroBloodnet , ePAGs and the Hematology ePAG Project Management Office has been working very closely with the EHA congress office in the organization of the "EHA Capacity Building Meeting"within the 23rd EHA congress.

Results

The 23rd EHA congress was held in Stockholm from June 14-17, 2018. The Capacity Building meeting was held on Thursday, the 14th of June 2018, between 2 pm and 5 pm. This session was mandatory for all patient advocates who received the EHA Fellowship.

The plenary session agenda:

- EuroBloodNet and the ePAG (Ananda Plate, ePAG representative)
- Getting the most out of EHA as a patient advocate (Jan Geissler, CML Advocates Network, ePAG representative)
- Introduction to PRO-Tools and QoL-instruments (Fabio Efficace, GIMEMA)
- Reading a scientific poster (Tamas Bereczky, EUPATI)
- Patient engagement in scientific publications (Dawn Lobban, Envision)

The meeting was successfully held with a total of 69 attendants.

Next steps

- Ongoing collaboration between ERN-EuroBloodNet ePAGs and EHA for the ”EHA Cpacity Building Meeting” within the 24th EHA Congress that will be held in Amsterdam from 13th to 16th of June 2019

3- Expected outcomes

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 deeper analyse the state of the art on the educational area of RHD by the compilation of the existing educational material for health professional and patients and the identification of the educational requirements for health professional and patients.

In turn, the analysis of the educational needs for professionals and patients has provided the evidence base required for defining the strategic plan and starting specific targeted-actions to address gaps that will continue during the upcoming years of implementation with the following expected long-term results:

- The facilitation of the harmonization of the haematology and paediatric (haematology) specialities curricula throughout the EU - European haematology curriculum/passport.
- The improvement of continuing medical education on ultra-rare haematological diseases among health professionals in UE.
- In line with the TFA Best practices: the promotion of diffusion and implementation of existing guidelines in the diagnosis and clinical management of ultra-rare haematological diseases, as well as existing recommendations in the field.

Annex I

List of ERN-EuroBloodNet experts responders to the Questionnaire on CME

List of ERN-EuroBloodNet who participated in the Questionnaire on CME

BE	Dominique Bron, Jules Bordet Institute
BE	Sebastian Wittnebel, Jules Bordet Institute
CY	Soteroula Christou, Archbishop Makarios III Hospital
CZ	Jiri Mayer, University Hospital Brno
CZ	Michael Doubek, University Hospital Brno
ES	Cristina Diaz de Heredia, Hospital Universitari Vall d'Hebron
ES	David Beneitez, Hospital Universitari Vall d'Hebron
FR	Isabelle Thuret, Assistance Publique-Hôpitaux de Marseille
FR	Maryse Etienne-Julan, CHU de Pointe-à-Pitre/Abymes
FR	Marie Petras, CHU de Pointe-à-Pitre/Abymes
FR	Pablo Bartolucci, Assistance Publique-Hôpitaux de Paris, Hôpital Henri-Mondor
IT	Achille Iolascon, AOU Federico II - Naples
IT	Nicoletta Masera, S. Gerardo Hospital - Monza
IT	Anna Falanga, Hospital Pope John XXIII - Bergamo
IT	Luca Barcella, Hospital Pope John XXIII - Bergamo
IT	Alessandro Vannucchi, AOU Careggi, Florence
IT	Domenico Girelli, AOUI Verona
IT	Lucia De Franceschi, AOUI Verona
IT	Carlo Dufour, IRCCS Institute Giannina Gaslini - Genoa
IT	Fiorina Giona, AOU Policlinico Umberto I - Rome
NL	Bart Biemond, Academic Medical Center Amsterdam
SE	Eva Hellström-Lindberg, Karolinska University Hospital
UK	Michael Makris, Sheffield Teaching Hospitals NHS Foundation Trust
UK	Baba Inusa, Guy's and St Thomas' NHS Foundation Trust
UK	Jo Howard, Guy's and St Thomas' NHS Foundation Trust
UK	Paul Telfer, Barts Health NHS Trust
UK	Banu Kaya, Barts Health NHS Trust

Annex II

List of educational material

Educational material for Red blood cell disorders						Rare Red blood cell defects								
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Haemoglobinopathy	SCD	Thal	Hereditary erythroenzymopathies	PKD	G6PD	Hereditary RBC membrane defects	Hereditary Spherocytosis	Congenital Erythrocytosis
1	Blood Diseases Introduction	A- Teaching material	A- Available in my Center	English	PPT									
2	Thalassaemias	A- Teaching material	A- Available in my Center	English	PPT	x		x						
3	Sickle cell disease	A- Teaching material	A- Available in my Center	English	PPT		x							
4	Iron Homeostasia 1	A- Teaching material	A- Available in my Center	English	PPT	x								
5	Iron Homeostasia 2	A- Teaching material	A- Available in my Center	English	PPT	x								
6	Gene and Cell Therapies	A- Teaching material	A- Available in my Center	English	PPT	x	x	x						
7	Prenatal Diagnosis 1	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
8	Prenatal Diagnosis 2	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
9	Bioinformatics 1	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
10	Bioinformatics 2	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
11	Pharmacogenetics & Pharmacogenomics	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
12	High Throughput Methods	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
13	NIPD for Single Gene Disorders	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
14	Preimplantation Genetic Diagnosis (PGD)	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
15	ITHANET Haemoglobinopathies specific Portal	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
16	Rare Anaemias Disorders European Epidemiological Platform (RADEEP)	A- Teaching material	A- Available in my Center	English	PPT	x	x	x	x	x	x	x	x	x
17	Introduction on erythropoiesis	A- Teaching material	A- Available in my Center	Greek	PPT	x		x						
18	Introduction on haemoglobinopathies	A- Teaching material	A- Available in my Center	Greek	PPT	x		x						
19	Management of Haemoglobinopathies	A- Teaching material	A- Available in my Center	Greek	PPT	x		x						

Educational material for Red blood cell disorders						Rare Red blood cell defects								
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Haemoglobinopathy	SCD	Thal	Hereditary erythroenzymopathies	PKD	G6PD	Hereditary RBC membrane defects	Hereditary Spherocytosis	Congenital Erythrocytosis
20	Manaegement of SCD	A- Teaching material	A- Available in my Center	Greek	PPT	x	x							
21	Chelation Therapy	A- Teaching material	A- Available in my Center	Greek	PPT	x		x						
22	Blood transfusion therapy	A- Teaching material	A- Available in my Center	Greek	PPT	x		x						
23	Management of pregmancies in thalassaemia	A- Teaching material	A- Available in my Center	Greek	PPT	x		x						
24	Case report studies	A- Teaching material	A- Available in my Center	Greek	PPT	x		x						
25	Chelation Therapy	B- Patient Education	A- Available in my Center	Greek	PDF	x		x						
26	Live with thalassaemia	B- Patient Education	A- Available in my Center	Greek	PDF	x		x						
27	Blood transfusion therapy	B- Patient Education	A- Available in my Center	Greek	PDF	x		x						
28	Thalassaemia info for patients	B- Patient Education	A- Available in my Center	Greek and English	PDF	x		x						
29	Can Thalassaemia be prevented	B- Patient Education	A- Available in my Center	Greek	PDF	x		x						
30	Guía de práctica clínica sobre enfermedad de células falciformes pediátrica SEHOP (Sociedad Española de Hematología y Oncología Pediátrica)	A- Teaching material	B - External material	Spanish	PDF		x							
31	Guía de manejo de las enfermedades falciformes SEHH (Sociedad española de Hematología y Hemoterapia)	A- Teaching material	B - External material	Spanish	PDF		x							
32	Guía de práctica clínica de la talasemia mayor e intermedia en pediatría SEHOP (Sociedad Española de Hematología y Oncología Pediátrica)	A- Teaching material	B - External material	Spanish	PDF			x						
33	El déficit de glucosa 6 fosfato deshidrogenasa. El favismo (SEHH)	B- Patient Education	B - External material	Spanish	PDF						x			
34	Rasgo falciforme	B- Patient Education	B - External material	Spanish	PDF		x							
35	Alfa talasemia	B- Patient Education	B - External material	Spanish	PDF			x						
36	Beta talasemia	B- Patient Education	B - External material	Spanish	PDF			x						
37	Enfermedad de células falciformes	B- Patient Education	B - External material	Spanish	PDF		x							
38	La aventura de la drepanocitosis	B- Patient Education	B - External material	Spanish	booklet		x							

Educational material for Red blood cell disorders						Rare Red blood cell defects								
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Haemoglobinopathy	SCD	Thal	Hereditary erythroenzymopathies	PKD	G6PD	Hereditary RBC membrane defects	Hereditary Spherocytosis	Congenital Erythrocytosis
39	La aventura de la talasemia	B- Patient Education	B - External material	Spanish	booklet			x						
40	La transfusión. El viaje de la sangre	B- Patient Education	B - External material	Spanish	booklet									
41	Pedro y Bill Bong. Drepanocitosis y transfusión	B- Patient Education	B - External material	Spanish	booklet		x							
42	National protocol of diagnosis and care for a rare disease: Major and intermediate thalassemia syndromes, coordinated by thalassemia reference center (Marseille)	A- Teaching material	B - External material	French	https://www.has-sante.fr/portail/upload/docs/application/pdf/2008-			x						
43	National Diagnostic and Care Protocol for a Rare Disease: Glucose-6-Phosphate Dehydrogenase (G6PD) Deficiency, coordinated by thalassemia reference center (Marseille)	A- Teaching material	B - External material	French	https://www.has-sante.fr/portail/upload/docs/application/p						x			
44	Recommendations for hematopoietic stem cell transplants in beta-thalassemia, coordinated by thalassemia reference center and SFGM-TC	A- Teaching material	B - External material	French	http://fr.ap-hm.fr/sites/default/files/files/crmr/recom			x						
45	Orphanet emergencies: Thalassemia, coordinated by thalassemia reference center (Marseille)	A- Teaching material	B - External material	French	https://www.orpha.net/data/patho/FR/Urgences_Thala			x						
46	Complex diagnostics in hemoglobinopathies, International Journal of Laboratory Hematology; Juin 2014	A- Teaching material	B - External material	French		x		x						
47	Evaluation of iron overload by MRI in polytransfused anemias: a guide for radiologists; January 2017 coordinated by thalassemia reference center (Marseille)	A- Teaching material	B - External material	French	http://fr.ap-hm.fr/sites/default/files/files/crmr/brochure_IRM_Foie_C	x		x						
48	Emergency and healthcare card ORPHANET: thalassemia coordinated by thalassemia reference center (Marseille)	A- Teaching material	B - External material	French	https://solidarites-sante.gouv.fr/IMG/pdf/DGS-			x						
49	Subcutaneous perfusion of desferal explained to patients and caregivers, produced by thalassemia reference center (Marseille)	B- Patient Education	Both	French	http://fr.ap-hm.fr/sites/default/files/files/crmr/perfusi			x						
50	Osteoporosis and thalassemia produced by thalassemia reference center (Marseille)	B- Patient Education	Both	French	http://fr.ap-hm.fr/sites/default/files/files/crmr/brochur			x						
51	Care pathway: adults and children produced by thalassemia reference center (Marseille)	B- Patient Education	Both	French				x						
52	Thalassemia and therapeutic patient education	B- Patient Education	Both	French				x						
53	Thalassemia reference center brochure	B- Patient Education	Both	French				x						
54	I have a beta thalassemia trait produced by thalassemia reference center (Marseille)	Both	Both	French	http://fr.ap-hm.fr/sites/default/files/files/crmr/beta_th			x						
55	The cartoon leila and marco over the days: Thalassemia, produced by thalassemia reference center (Marseille)	B- Patient Education	Both	French				x						

Educational material for Red blood cell disorders						Rare Red blood cell defects								
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Haemoglobinopathy	SCD	Thal	Hereditary erythroenzymopathies	PKD	G6PD	Hereditary RBC membrane defects	Hereditary Spherocytosis	Congenital Erythrocytosis
56	Dietetic advice in thalassemia: food iron, produced by thalassemia reference center (Marseille)	B- Patient Education	Both	French	http://fr.ap-hm.fr/sites/default/files/files/			x						
57	National Diagnostic and Care Protocol for a Rare Disease: Adult Sickle Cell Disease Syndromes, collaboration of thalassemia reference center (Marseille)	A- Teaching material	B - External material	French	https://www.has-sante.fr/portail/upload/docs		x							
58	National Diagnostic and Care Protocol for a Rare Disease: Major Sickle Cell Disorders in Children and Adolescents, collaboration thalassemia reference center (Marseille)	A- Teaching material	B - External material	French	https://www.has-sante.fr/portail/upload/docs		x							
59	Encyclopedia Orphanet General Public : Beta-thalassemia, coordinated by Orphanet and thalassemia reference center (Marseille)	B- Patient Education	B - External material	French	https://www.orpha.net/data/patho/Pub/fr/BetaThalass			x						
60	Encyclopedia Orphanet General Public : Alpha-thalassemia, coordinated by Orphanet and thalassemia reference center (Marseille)	B- Patient Education	B - External material	French	https://www.orpha.net/data/patho/Pub/fr/AlphaThalas			x						
61	DREPACURE	B- Patient Education	Both											
62	CARTES A THEME	B- Patient Education	Both											
63	PHOTOLANGAGE	B- Patient Education	B - External material											
64	National Reccomandation on SCD	A- Teaching material	A- Available in my Center	Italian	http://www.site-italia.org/colla		x							
65	Interactive algorithm for the managemt of acute events in SCD	A- Teaching material	A- Available in my Center	Italian	pdf1		x							
66	App for managemnt of main queries on G6PD	Both	A- Available in my Center	Italian and English	http://www.site-italia.org/colla						x			
67	Depliant on SCD developed with patient association	B- Patient Education	A- Available in my Center	Italian and English	pdf2		x							
68	Depliant on Thalassemia developed with patient association	B- Patient Education	A- Available in my Center	Italian and English	pdf3			x						
69	Nathan and Osky's Hematology of infancy and childhood	A- Teaching material	A- Available in my Center	English	textbook	x	x	x	x	x	x	x	x	x
70	ANEMIE EREDITARIE (RDG010) PERCORSO DIAGNOSTICO TERAPEUTICO ASSISTENZIALE	Both	Both	Italian	http://www.regione.lazio.it/malattierare/	x	x	x				x	x	
71	Patologia del Globulo Rosso	B- Patient Education	B - External material	Italian	http://www.aieop.org/web/famiglie/#sch		x	x			x		x	x
72	Academy for sickle cell and thalassaemia (ASCAT) , annual conference	Both	B - External material	English	www.scorecharity.com	x	x	x			x			
73	south thames sickle cell and thalassaemia network, UK	Both	Both	English	www.ststn.co.uk	x	x	x		x	x	x	x	
74	UK Forum scientific and educational meetings	Both	Both	english	www.haemoglobin.org.uk	x	x	x	x	x	x	x	x	
75	Evelina London Children's Hospital	Both	A- Available in my Center	english	www.evelinalondon.nhs.uk	x	x	x			x		x	
76	A parent's guide to managing sickle cell disease	B- Patient Education	Both	English and French translation	https://www.gov.uk/government/publicat	x	x							

Educational material for Red blood cell disorders						Rare Red blood cell defects								
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Haemoglobinopathy	SCD	Thal	Hereditary erythroenzymopathies	PKD	G6PD	Hereditary RBC membrane defects	Hereditary Spherocytosis	Congenital Erythrocytosis
77	Adult carrier leaflets: sickle cell, thalassaemia, unusual haemoglobin	Both	Both	English and translations available to download	https://www.gov.uk/government/collectio	x	x							
78	Screening Tests for you and your baby	Both	Both	English and translations available to download (Arabic,	https://www.gov.uk/government/publicat	x	x							
79	Standards for sickle cell and thalassaemia screening	A- Teaching material	Both	English	https://www.gov.uk/government/publicat	x	x							
80	NHS Sickle Cell and Thalassaemia Screening Programme Handbook	A- Teaching material	Both	English	https://www.gov.uk/government/publicat	x	x							
81	NHS Sickle Cell and Thalassaemia Screening Programme Laboratory Handbook	A- Teaching material	Both	English	https://www.gov.uk/government/publicat	x	x							
82	SCT Antenatal and PND care pathways	A- Teaching material	Both	English	https://www.gov.uk/government/publicat	x	x							
83	Family Origin Questionnaire	A- Teaching material	Both	English	https://www.gov.uk/government/publicat	x	x							
84	Health professional guidance and template letters for contacting parents regarding antenatal screening, prenatal diagnosis and newborn screening	A- Teaching material	Both	English	https://www.gov.uk/government/collections/sickle-cell-	x	x							
85	Guidance and information on quality assurance and QA processes for SCT screening, data collection, equitable access and transition from screening into care	A- Teaching material	Both	English	https://www.gov.uk/government/collections/sickle-cell-	x	x							
86	Updates regarding education and training courses for health professionals	A- Teaching material	Both	English	https://www.gov.uk/guidance/sickle-cell-and-	x	x							
87	Sickle Cell Disease in Clinical Practice. Howard J and Telfer P Springer 2015	A- Teaching material	B - External material	English		x	x							
88	UK Thalassaemia Society Standards for the Clinical Care of Children and Adults with Thalassaemia in the UK. 3rd Edition 2016	A- Teaching material	B - External material	English	Available on UKTS website	x		x						
89	UK Sickle Cell Society Standards for the Clinical Care of Adults with Sickle Cell Disease in the UK. 2nd Edition 2018	A- Teaching material	B - External material	English	Available on SCS WEBSITE	x	x							
90	Royal London Hospital edcuational programme in haemoglobinopathies. Different teaching programmes for haematologists in training, paediatricians in training, nurse specialists and medication students	A- Teaching material	A- Available in my Center	English	Contact Dr P Telfer and Dr B Kaya	x	x	x						
91	Telfer P. Sickle Cell Pain (005-0036). e-Pain. HEE e-Learning for Healthcare. 2016.	A- Teaching material	B - External material	English	Through NHS e-learning website	x	x							
92	A parent's guide to managing sickle cell disease	B- Patient Education	Both	English and French translation	https://www.gov.uk/government/publications/sickle-cell-		x							
93	Adult carrier leaflets: sickle cell, thalassaemia, unusual haemoglobin	Both	Both	English and translations available to download	https://www.gov.uk/government/collections/adult-		x	x						
94	Screening Tests for you and your baby	Both	Both	English and translations available to download (Arabic, Bengali, simplified	https://www.gov.uk/government/publications/screenin									

Educational material for Red blood cell disorders						Rare Red blood cell defects								
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Haemoglobinopathy	SCD	Thal	Hereditary erythroenzymopathies	PKD	G6PD	Hereditary RBC membrane defects	Hereditary Spherocytosis	Congenital Erythrocytosis
95	Standards for sickle cell and thalassaemia screening	A- Teaching material	Both	English	https://www.gov.uk/government/publications/standard		x	x						
96	NHS Sickle Cell and Thalassaemia Screening Programme Handbook	A- Teaching material	Both	English	https://www.gov.uk/government/publications/handboo		x	x						
97	NHS Sickle Cell and Thalassaemia Screening Programme Laboratory Handbook	A- Teaching material	Both	English	https://www.gov.uk/government/publications/sickle-cell-		x	x						
98	SCT Antenatal and PND care pathways	A- Teaching material	Both	English	https://www.gov.uk/government/publications/sickle-cell-									
99	Family Origin Questionnaire	A- Teaching material	Both	English	https://www.gov.uk/government/publications/family-									
100	Health professional guidance and template letters for contacting parents regarding antenatal screening, prenatal diagnosis and newborn screening	A- Teaching material	Both	English	https://www.gov.uk/government/collections/sickle-cell-									
101	Guidance and information on quality assurance and QA processes for SCT screening, data collection, equitable access and transition from screening into care	A- Teaching material	Both	English	https://www.gov.uk/government/collections/sickle-cell-									
102	Updates regarding education and training courses for health professionals	A- Teaching material	Both	English	https://www.gov.uk/guidance/sickle-cell-and-									

Educational Material for Bone Marrow Failures						Bone marrow failure (BMF) and rare haematopoietic disorders							
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Congenital dyserythropoietic anemia	Blackfan-Diamond anemia	Aplastic anemia	PNH	GATA2 syndrome	Fanconi Anemia	Transient erythroblastopenia of childhood	Other
1	Neutropenia crónica grave. Guía de diagnóstico, tratamiento y seguimiento SEHOP	A- Teaching material	B - External material	Spanish	PDF								x
2	Nathan and Osky's Hematology of infancy and childhood	A- Teaching material	A- Available in my Center	English	Textbbok	x	x	x	x	x	x	x	Severe Congenital Neutropenia, Telomere Biology Diseases,
3	Congenital & Acquired Bone Marrow failure, M al Jurf, E Gluckman, C Dufour Editors, cademic press, Elsevier 2017	A- Teaching material	A- Available in my Center	English	Textbook	x	x	x	x	x	x	x	x
4	How I manage Fancon Anemia , Br J Haematol. C Dufour 2017 Jul;178(1):32-47	A- Teaching material	A- Available in my Center	Emglish	Review						x		
5	Classical inherited bone marrow failure syndromes with high risk for myelodysplastic syndrome and acute myelogenous leukemia. Svage SA & Dufour C, Semin Hematol. 2017 Apr;54(2):105-114	A- Teaching material	A- Available in my Center	English	Review		x			x	x		Telomere Biology Diseases, Severe Congenital neutropenias
6	How I treat MDS and AML in Fanconi anemia. Blood. 2016 Jun 16;127(24):2971-9, R.Peffault de latour & J Soullier	A- Teaching material	A- Available in my Center	English	Review						x		
7	The diagnosis and Treatment of Aplstic Anemia, Miano M & Dufour C , Int J Hematol. 2015 Jun;101(6):527-35	A- Teaching material	A- Available in my Center	English	review			x					
8	How I treat acquired aplastic anemia. A.Bacigalupo, Blood. 2017 Mar 16;129(11):1428-1436S The diagnosis and treatment of aplastic anemia: a review.	A- Teaching material	A- Available in my Center	English	Review			x					
9	Anemia aplastica acquisita · aplasia midollare · aplasia midollare acquisita · malattie rare .... in diagnosi differenziale quali le leucemie, le mielodisplasie (MDS) ipocellulari, le aplasie nell'ambito di malattie autoimmuni, ... Mattioli HEALTH	Both	A- Available in my Center	Italian	https://www.ematologiainprogress.it/aplasia-midollare-acquisita/			x					
10	Pearson syndrome. Expert Rev Hematol. 2018 Mar;11(3):239-246, Farruggia P, Di Marco F, Dufour C	A- Teaching material	A- Available in my Center	English	Review								Perason Syndrome

Educational Material for HH-iron						Hemochromatosis and other rare genetic disorders of iron metabolism and heme synthesis					
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	non-HFE related hereditary hemochromatosis: (HH types 2A, 2B, 3 and 4A,4B), TFH1-Related Hemochromatosis (type V), Hereditary Hyperferritinemia Cataract Syndrome (HHCS)	HFE-related hereditary hemochromatosis with established severe clinical expression or due to very rare mutations in HFE	Low iron availability for erythropoiesis: Iron Refractory Iron deficiency Anemia (IRIDA), Aceruloplasminemia (ACP)	Rare defects in iron acquisition and transport: Atransferrinemia, Microcytic anemia with iron loading (DMT1), Sideroblastic anemia (STEAP3)	Defects in heme synthesis or Fe-S cluster biogenesis: Sideroblastic anemias (SLC25A38, GLRX5; HSPA9), XLSA with ataxia (ABCB7), XLSA (ALAS2)	Other
1	PDTA EMOCROMATOSI EREDITARIA	A- Teaching material	B - External material	Italian	http://malattierare.marionegri.it/images/downloads/PDTA/PDTA_schede/emocromatosi_ereditaria.pdf	X	X	X	X		
2	PDTA ACERULOPLASMINEMIA CONGENITA	A- Teaching material	B - External material	Italian	http://malattierare.marionegri.it/images/downloads/PDTA/PDTA_schede/aceruloplasminemia_congenita.pdf			X			
3	Associazione per lo Studio dell'Emocromatosi e delle Malattie da Sovraccarico di Ferro ONLUS WEBSITE	Both	B - External material	Italian	https://www.emocromatosi.it/articoli.htm	X	X	X			
4	VARIOUS EDUCATIONAL MATERIAL REGARDING SPECIFIC DISEASES	Both	B - External material	Italian, English, French and other languages	www.orpha.net	X	X	X	X	X	
5	Website of Interdisciplinary Group of Iron Disorders of Verona	Both	A- Available in my Center	Italian (an English translation of some parts is provided)	www.gimferverona.it	X	X	X	X	X	All types of iron disorders (rare and common)
6	Brochure for patients with Hereditary Hemochromatosis	B- Patient Education	A- Available in my Center	Italian and English	PDF	X	X				All types of HH (HFE and non-HFE)
7	PPT Slides on Gaucher Disease as a rare cause of iron disorder	A- Teaching material	A- Available in my Center	Italian	PDF						Gaucher Disease (possibly associated to hyperferritinemia)
8	PPT Slides on Next Generation Sequencing for Diagnosis of Atypical Genetic Iron disorders	A- Teaching material	A- Available in my Center	English	PDF	X					
9	Nathan and Osky's Hematology of infancy and childhood	A- Teaching material	A- Available in my Center	English							

Educational Material for bleeding-coagulation disorders						Rare bleeding-coagulation disorders: which diseases are covered by the eductaional material? Please, tick all corresponding cells					
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Haemophilia A and B (including female carriers)	The rarer congenital deficiencies of other coagulation factors (such as fibrinogen and factors II, V, VII, X, XI and XIII)	Von Willebrand disease	Rare hemorrhagic disorder due to a platelet anomaly	thrombotic thrombocytopenic purpura	Rare thrombotic disorder due to a platelet anomaly
1	PDTA DISORDINI EREDITARI EMORRAGICI	A- Teaching material	B - External material	Italian	http://malattierare.marionegri.it/images/downloads/PDTA/PDTA_schede/disordini_ereditari_emorragici.pdf	X	X	X			
2	PDTA PORPORA TROMBOTICA TROMBOCITOPENICA	A- Teaching material	B - External material	Italian	http://malattierare.marionegri.it/images/downloads/PDTA/PDTA_schede/porpora_trombotica_trombocitopenica.pdf					X	
3	PORPORA TROMBOTICA TROMBOCITOPENICA	B- Patient Education	B - External material	Italian	http://malattierare.marionegri.it/images/pdf_schede_malattie/P_T/porpora_trombotica_trombocitopenica.pdf					X	
4	VARIOUS EDUCATIONAL MATERIAL REGARDING THE SPECIFIC DISEASES	Both	B - External material	Italian, English, French and other languages	www.orpha.net	X	X	X	X	X	X

Educational Material for Lymphoid malignancies						Lymphoid malignancies: which diseases are covered by the eductaional material?											
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Acute lymphoblastic leukemia (ALL)	Marginal zone lymphomas	mantle cell lymphoma	peripheral T-cell lymphoma	Hairy cell leukaemia	Light chain Amyloidosis (AL amyloidosis)	Diffuse large B-cell lymphoma (DLBCL)	Follicular lymphoma	Chronic Lymphocytic Leukemia (CLL)	Hodgkin lymphoma	Waldenström macroglobulinemia	Other
1	BHS national courses	A - Teaching material	A - Available in my Center (members only)	English	BHS.be/education	x	x	x	x	x	x	x	x	x	x	x	All myeloid malignancies
2	EHA e-learning center	A - Teaching material	A - Available in my Center (members only)	English	learningcenter.ehaweb.org	x	x	x	x	x	x	x	x	x	x	x	
3	EBMT e-learning	A - Teaching material	A - Available in my Center (members only)	English	ebmt.org/education												Focused on transplantation
4	European School of Hematology	A - Teaching material	A - Available in my Center (members only)	English	elearning.esh.org	x	x	x	x	x	x	x	x	x	x	x	
5	LYSA website	A - Teaching material	B- External material (members only)	French	lysa-lymphoma.org		x	x	x			x	x		x		focused on lymphomas
6	LYSA website	B - patient education	B - External material (for all patients)	French													
7	Chronická lymfocytární leukémie, Informace pro pacienty a jejich blízké	B - patient education	A- Available in my Center	Czech	Printed form and PDF									x			
8	Akutní lymfoblastická leukémie, Informace pro pacienty a jejich blízké	B - patient education	A- Available in my Center	Czech	Printed form and PDF	x											
9	MASTER UNIVERSITARIO DI II LIVELLO IN " MALATTIE RARE"	A - Teaching material	A- Available in my Center	Italian	https://www.uniroma1.it/it/offerta-formativa/mast	x									x	x	

Educational Material for Myeloid disorders						Myeloid malignancies									
nº	Title of the educational material	Indicate if A- Teaching material or B- Patient Education	Indicate if A- Available in your centre or B - External material	Language/s of the material available	Link or indicate annex (PDF, PPT)	Myelodysplastic syndrome (MDS)	Acute myeloid leukemia (AML)	Acute promyelocytic leukemia	Chronic myelomonocytic leukemia (CMML)	Chronic Myeloid Leukemia (CML)	Myeloproliferative neoplasm (MPN)	Myelofibrosis	Systemic mastocytosis	Histiocytosis	Other
1	Akutní myeloidní leukémie	B - patient education	A- Available in my Center	Czech	print form and PDF		x								
2	Chronická myeloidní leukémie	B - patient education	A- Available in my Center	Czech	print form and PDF					x					
3	Polycythemia Vera	B - patient education	A- Available in my Center	Italian							x				
4	Myelofibrosis	B - patient education	A- Available in my Center	Italian								x			
5	Essential Thrombocythemia	B - patient education	A- Available in my Center	Italian							x				
6	Brochure for patients with Mastocytosis	B - patient education	A- Available in my Center	Italian	PDF (5-opuscolo-per-il-paziente-con-mastocitosi_ASIMAS_ver								x		
7	Leucemia mieloide cronica	B-patient education	B- external material	Italian	www.ail.it/patologie-e-terapie/patologie-ematologiche/leucemia/					x					
8	Sindromi mielodisplastiche	B-patient education	B- external material	Italian	www.ail.it/patologie-e-terapie/patologie-ematologiche/leucemia/	x									
9	Gestione delle complicanze durante il trattamento di induzione nella leucemia acuta promielocitica	A-teaching material	B- external material	Italian	www.accmed.org/scheda?id_scheda=1270&area_scheda=AF			x							
10	Cardio-oncologia: gestione integrata del paziente con LMC	A-teaching material	B- external material	Italian	http://www.fadecm.net/corsi-fad-2018/lezioni-cardio-oncologiagestione-					x					
11	Identification and assessment of frailty in older patients with chronic myeloid leukemia and myelofibrosis, and indications for TKI treatment	A-teaching material	B- external material	english	www.ncbi.nlm.nih.gov/pubmed/29468276					x	x	x			
12	Interviste su LMC	B-patient education	B- external material	Italian	www.youtube.com/user/AssociazioneAIL/videos?disable_polymer=1					x					
13	ISTIOCITOSI CRONICHE PERCORSO DIAGNOSTICO TERAPEUTICO ASSISTENZIALE	A-teaching material and B-patient education	A- Available in our Centre and B- external material	Italian	http://www.regione.lazio.it/malattierare/allegati/PDTA/RCG150_istiocitos									x	
14	Malattie rare e Istiocitosi a cellule di Langerhans	A-teaching material	B- external material	Italian	https://www.ematologiainprogress.it/le-istiocitosi/									x	
15	Malattia di Erdheim-Chester	A-teaching material	B- external material	Italian	https://www.ematologiainprogress.it/malattia-di-erdheim-chester/									x	
16	La preservazione della fertilità in Oncoematologia	A-teaching material	B- external material	Italian	http://www.gemmedormienti.org/convegno-la-preservazione-della-										x
17	"ISTIOCITOSI A CELLULE DI LANGERHANS"	B-patient education	B- external material	Italian	See attached file 1									x	
18	MASTER UNIVERSITARIO DI II LIVELLO IN " MALATTIE RARE"	A-teaching material	A- Available in our Centre	Italian	https://www.uniroma1.it/it/offerta-formativa/master/2018/	x	x	x	x	x	x	x		x	

Annex III

AA and PNH Precerptoship program

1st Preceptorship University of Naples Federico II, Naples, Italy

Teachers

- Prof. Antonio M. Risitano (coordinator)
- Dr. Maddalena Raia
- Dr. Serena Marotta

Topic

Acquired bone marrow failure syndromes: aplastic anemia and paroxysmal nocturnal hemoglobinuria from the bench to the bedside

Description

This course is organized in a 3-day program which will include sessions of different format, such as formal lectures, informal discussion, case study discussion, and direct tutorial activity (in the clinic and/or in a lab). The course aims to discuss AA, PNH, and other related bone marrow failure syndromes, starting from their biology (similarities and gaps will be illustrated) to better describe clinical manifestations. AA and PNH as clinical entities will be discussed in detail, to explain the current treatment algorithms and the original data from where they were generated. Given the purpose of the preceptorship, the discussion will be oriented toward current clinical questions, and possible ways to make the clinical management of these diseases more effective in a world-wide scale. Indeed, ongoing researches aiming to improve treatment outcome will be presented, to make the audience aware about the future directions in the field.

Objectives

1. To improve the understanding of pathogenic mechanisms underlying AA and PNH
2. To share diagnostic algorithm to dissect AA and PNH in the context of the broad field of acquired and inherited bone marrow failure syndromes
3. To inform about current treatments and guidelines in AA and PNH, included (but not limited):
    a. Bone marrow transplantation
    b. Immunosuppressive treatment(s)
    c. Anti-complement treatment(s)
4. To foster the discussion about cutting-edge treatment strategies which may emerge as novel standard of care for AA and PNH

Agenda

	DAY	Type	Duration	Time	Topic(s)
Session 0	1	Welcome	0,5 hours	8,30-9	Presentation of the course
Session 1	1	Lectures	2 hours	9-11	The biology of bone marrow failure syndromes; 3 lectures on: – The pathophysiology of AA – The pathophysiology of PNH – AA and PNH: friends of foes?
Session 2	1	Round table	2 hours	11,30-13,30	Discussion of the topics presented in session 1, focusing on pathogenesis and diagnostic algorithm of AA and PNH
Session 3	1	Laboratory tutorial	3 hours	14,30-17,30	The differential diagnosis of bone marrow failure syndrome – focus on flow cytometry
Session 4	2	Lectures	2 hours	9-11	The treatment of bone marrow failure syndromes; 3 lectures on: – Bone marrow transplantation – Immunosuppression – Anti-complement treatment
Session 5	2	Round table	2 hours	11,30-13,30	Discussion of the topics presented in session 4, focusing on the treatment algorithm of A and PNH
Session 6	2	Case studies	3 hours	14,30-17,30	Critical review of patients' medical record to discuss real-word management of AA and PNH
Session 7	3	Clinical tutorial	4 hours	9-13	Clinical round in the clinical unit
Session 8	3	Lectures	2 hours	14-16	Future strategies to improve current treatment of AA and PNH
Session 9	3	Round table	1 hour	16-17	Discussion about gaps between ongoing research and real-life management
Session 10	3	Round table	0,5 hours	17-17,30	Take home message: where are we going?

2nd Preceptorship : Pediatric Hospital - IRCCS Institute Giannina Gaslini, Genoa, Italy.

Teacher

Carlo Dufour , Chair Hematology Unit, G . Gaslini Childrens' Hospital

Topic

Inherited and acquired bone marrow failures including AA and PNH in children and adolescents

Description

The spectrum of marrow failure syndromes in pediatric ages has a different composition compared to adults because of a larger proportion of inherited forms sometimes with masked or misleading phenotype. Also AA and PNH show some difference as for approach and outcome respect to adult forms. Based on this it is important to provide applicants with the fundamental tools for a correct diagnostic and treatment approach to marrow failures, including AA and PNH in children and adolescents .

Objectives

1. To learn differential diagnosis algorithm of marrow failure syndromes in children and adolescents with a special focus on atypical/ attenuated forms and on and PNH.

2. To learn pediatric treatment algorithm for AA, PNH and for the most frequent inherited bone marrow failure syndromes.

3. To learn pre/post treatment monitoring schedules s of pediatric patients with marrow failure including AA and PNH.

Agenda

Session 1	DAY1 Interactive lectures on diagnosis of Inherited and acquired bone marrow failures including AA and PNH-. optionally also in AHES- in children and adolescents	DAY2 Tutorials on clinical cases of AA, PNH, and AHES (the latter optionally)	DAY 3 Attendance to ward round and/or outpatient of AA/PNH and other constitutional marrow failures	Day 4 Specific session to discuss with/ask teachers selected topic raised by participants on issue faced in previous days
Session 2	Interactive lectures on treatment and long term management of Inherited and acquired bone marrow failures including AA and PNH- optionally also in AHES- in children and adolescents	Tutorials on clinical cases of the main inherited marrow failure syndromes	Attendance to hematology diagnostic Lab (morphology, Flow cytometry, molecular biology)	Wrap- up and take home message session

3rd Preceptorship : AP-HP, Hôpital Saint-Louis, Paris, France

Teachers

Prof Régis Peffault De La Tour, AP-HP, Hôpital Saint-Louis, Service d'hématologie Greffe, Trèfle 3, Paris, France

Dr Flore Sicre de Fontbrune, AP-HP, Hôpital Saint-Louis, Service d'hématologie Greffe, Trèfle 3, Paris, France

Prof Jean Hugues Dalle, AP-HP, Hôpital Robert Debré, Service d'hématologie pédiatrique, Paris, France

Topic

Inherited and acquired bone marrow failures including AA and PNH in adult and pediatric patients.

Description

The spectrum of marrow failure syndromes in pediatric ages has a different composition compared to adults because of a larger proportion of inherited forms sometimes with masked or misleading phenotype. Also AA and PNH show some difference as for approach and outcome respect to adult forms. Based on this it is important to provide applicants with the fundamental tools for a correct diagnostic and treatment approach to marrow failures, including AA and PNH, in pediatric and adult patients.

Objectives

1. To learn differential diagnosis algorithm of marrow failure syndromes in adult and pediatric patients with a special focus on atypical/ attenuated forms and on PNH.

2. To learn adult treatment algorithm for AA, PNH and for the most frequent inherited bone marrow failure syndromes.

3. To learn pre/post treatment monitoring schedules s of adult patients with marrow failure including AA and PNH.

Agenda

Session 1	DAY1 Interactive lectures on diagnosis of Inherited and acquired bone marrow failures including AA and PNH-. optionally also in adult and pediatric patients	DAY2 Tutorials on clinical cases of AA, PNH, and AHES (the latter optionally)	DAY 3 Attendance to ward round and/or outpatient of AA/PNH and other constitutional marrow failures	Day 4 Specific session to discuss with/ask teachers selected topic raised by participants on issue faced in previous days
Session 2	Interactive lectures on treatment and long term management of Inherited and acquired bone marrow failures including AA and PNH- optionally also in AHES- in adult patients	Tutorials on clinical cases of the main inherited marrow failure syndromes	Attendance to hematology diagnostic Lab (morphology, Flow cytometry, molecular biology)	Wrap- up and take home message session