



DELIVERABLE 6.5 ERN-EUROBLOODNET REPORT ON ACTIONS FOR THE PROMOTION OF PATIENTS' EMPOWERMENT 2

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

DELIVERABLE 6.5 ERN-EUROBLOODNET REPORT ON ACTIONS FOR THE PROMOTION OF PATIENTS' EMPOWERMENT 2

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 Educational & Patients Program Manager

Victoria Gutierrez Valle – ERN-EuroBloodNet Dissemination & IT Manager

Maria Rodríguez Sánchez - ERN-EuroBloodNet Manager

Maria del Mar Mañú Pereira – ERN-EuroBloodNet Scientific Director

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

Pierre Fenaux - ERN-EuroBloodNet Coordinator and Oncological Hub chair

Short Description

Report on projects implemented for the promotion of patients' empowerment including: patients advocacy's actions, educational workshops and webinars, patient therapeutic education programs at International congress, cross border health patients assistance, and reporting of the collaboration with EURORDIS and European Patients Organizations for the reinforce of ERN-EuroBloodNet patient-centered actions.

Publication Date

28/02/2022

TABLE OF CONTENTS

1. Introduction

2. Patients Advocacy's actions implemented

- 2.1 Increasing of European Patients Groups Representation for Hematology
- 2.2 Patients' advocacy actions on ERN-EuroBloodNet's website & Supporting Patients' Organizations Profile
- 2.3 Facilitating the creation of a bottom up European Network of SCD Patients Organizations

3. European Cross Border Health Assistance to patients

- 3.1 EuroBloodNet Cross Border Health assistance to Rare Hematological Diseases patients, an infopoint for patients dealing with Cross Border Health Rights

4. Educational actions implemented

4.1 Sickle Cell Disease

- 4.1.1 Educational Patients session at congress: Sickle Cell Disease Patients Educational Session at ASCAT 2022 and 2022 January
- 4.1.2 ERN-EuroBloodNet participating to the World Sickle Cell Day 2020 and 2021
- 4.1.3 Sickle Cell Disease Patient Priority Blog
- 4.1.4 Pilot of Sickle Cell Disease Patients Conversation Group with a psychotherapist

4.2 Topic on Focus for Patients and their family or patients organizations

- 4.2.1. Topic on Focus on Cutaneous Lymphoma for patients associations
- 4.2.2 Topic on Focus on Myelodysplastic Syndromes for patients and their families
- 4.2.3 Topic on Focus on Sickle Cell Disease for patients and their families

4.3. Patients Webinars

- 4.3.1 COVID-19 and Sickle Cell Disease
- 4.3.2 Congenital Dyserythropoietic Anemia I and Congenital Dyserythropoietic Anemia II

5. Overview on forthcoming patients' project

6. Conclusions

Annexes

- Annex I ASCAT 2020
- Annex II ASCAT 2022 January

1. INTRODUCTION

With the creation of the European Reference Networks (ERNs), the EU has provided the requirement and the means for empowering patients' community, raising awareness and promoting therapeutic education on rare diseases for patients' organizations or patients and their families. In addition, ERN could provide a solid visibility to rare diseases patients' voice, bringing their needs and expectations to the attention of the public at large, scientific community and policymakers. Finally, ERNs could provide cross border health assistance to Rare Hematological Diseases Patients, as they are structured for knowledge sharing and coordination of health care across the European Union.

Knowledge on rare diseases and access to information could be scarce and not very well developed because of the scattered accessible evidence on those complex conditions and the difficulties of identifying patients' cohorts because of the low incidence of those diseases. This situation causes lack of healthcare services, inadequate disease management, poor presence of patients associations, inaccessibility to treatments and highly specialized procedures, insufficient availability of therapeutic educational material, and finally a consequent isolation of patients and patients' families.

The ERN-EuroBloodNet is playing a crucial role in Europe for improving clinical patient management and increasing patients and caregivers quality of life. Thanks to the close collaboration with expert HCPs, EURORDIS, ePAGS and European, National or Local patient's representatives, the ERN is achieving relevant outcomes.

In this deliverable it is redacted the report on actions and projects overtaken with those above-mentioned scopes. The solid plan carried by the ERN-EuroBloodNet focuses on: patients' advocacy's actions, promoting patient involvement in networks, organization of educational workshops and webinars, investing in patient therapeutic educational programs at International congress, providing cross border health patients assistance, improving health outcomes from the patient-centered approach and giving visibility to patients actions and patients organization collaboration on the Network's website.

During the third period of network implementation, ERN-EuroBloodNet has focused efforts on three axes that reflect the added values of the ERNs:

- Patients Advocacy: increase of ePAGs advocates representing the Network, provision of accessible information on the Network's website on existing European patients organizations, list of the collaboration of patient associations with the ERN and its related actions. ERN-EuroBloodNet's aim is to facilitate patients rights and access to information in order to have access to healthcare services regardless of a state's geographical boundaries or the ability of national health services to provide high quality services by increasing patients representation at European Level.
- European Cross Border Health Assistance: A central infopoint in Europe for assisting RHDs patients in accessing healthcare services across Member States in accordance with Directive 2011/24/EU.
- Promotion of Patients Education: producing educational offers addressed to RHD patients' community at large and patients' organisations. To understand a disease from a clinical and social point of view means to improve the quality of life of the patients themselves.

With respects to actions addressed to patients, we distinguish in this deliverable those that the ERN-EuroBloodNet targets for

1. Patients and their families:
 - a. Patients Webinars or Topic on Focus
 - b. Educational patients' session at congress
 - c. Rare Disease World Awareness Campaigns
 - d. Establishment of patients associations and advocacy trainings
2. Patients Advocates and Patients Organisations:
 - a. Collaboration with ePAGs and Eurordis on comprehensive actions of the ERN-EuroBloodNet
 - b. Topic on focus webinars for Patients Organisations
3. Both:
 - a. Repository of Educational Material for patients and patients organizations
 - b. Information on existing European patients' organizations, associations collaborating with the Network and its related actions.

Finally, the actions described are gathered:

1. Transversal to every RHDs: projects dedicated to patients advocacy and Cross Border Health related issues
2. Disease specific: projects addressed to Sickle Cell Disease and Cutaneous Lymphoma

The different actions reported include the following sections: Rationale, Objective, Methods, Results, Next step

2. PATIENTS ADVOCACY'S ACTIONS IMPLEMENTED

Introduction

Patients' organisations play a crucial role in healthcare on local, regional, national, European or International level through support groups or via website and social media. They are an info-point that provides valid documentation for helping patients and their family to know their condition and better manage the disease, giving a solid support to learn how to live with its symptoms and side effects. They also could work as helpdesk for assisting patients in navigating their healthcare systems or benefitting from cross border health rights when needed. Finally, patient advocacy organisations work with policy makers, healthcare institutions, clinicians, researchers and industry to make sure all health policy and research delivers to patients' needs and priorities and by representing the voice of patients affected by Rare diseases.

Recognizing how crucial is the operation of patients organizations, the ERN-EuroBloodNet has established a plan for supporting them, achieving good results. The ERN has strengthened its collaboration with national or European and International patients associations promoting a patient-centered approach to its projects and involving patients representatives development's tasks and board decisions making.

During last year, part of the Network's work has focused on giving more visibility to patients' associations' collaboration, by fulfilling the website with the description of the project undertaken, by identifying new cooperation with patients associations and trying to involve those on ERN's actions plan. Finally, the ERN has provided the means for balancing patients and clinical needs specifically to a disease's domain and strengthening the relationship between health professionals and patients.

2.1 INCREASING OF EUROPEAN PATIENTS GROUPS REPRESENTATION FOR HEMATOLOGY

Rationale

In parallel to the establishment of European Reference Networks (ERNs), EURORDIS - Rare Diseases Europe has initiated the creation of European Patient Advocacy Group (ePAG) for each ERN to bring together the patient organisations whose rare diseases are covered by a specific ERN such as EuroBloodNet. The ePAG Advocates are nominated to represent their disease area in the ERN EuroBloodNet as well as the interests of the wider patient community affected by rare hematological diseases.

ePAGs constitute one of the main cornerstones of the European Reference Networks. The involvement of patients' organizations in the Board of EuroBloodNet has been assured from the beginning in order to guarantee their pivotal role within the network and keep EuroBloodNet patients' centred approach. Their role could be invested in several domains:

- Ensuring patient-centred care and respect for patients' rights and choices
- Ensure transparency in quality of care, safety standards, clinical outcomes and treatment options
- Ensure that ethical aspects for patients are properly taken into account in balancing patient and clinical needs
- Contribute to the development of information for patients on health policies, good clinical practice, treatment pathways and dissemination of guidelines
- Contributing to the planning, monitoring and evaluation of ERN initiatives
- EuroBloodNet Cross Border Health assistance to Rare Hematological Diseases patients, an infopoint for patients dealing with Cross Border Health Rights.

Objective

The main objective fixed by the Network has been to increase the European patients' groups' representation for Hematology according to pre-identified gaps in the representation of not covered clinical areas

Methods

The nomination of ePAGs is regulated according to EURORDIS internal rules. It is fundamental the strategical and joint collaboration among ERN-EuroBloodNet coordination team and EURORDIS. The action consisted of two steps: identifying those clinical areas not yet covered by a European patient representative and secondarily together with EURORDIS identifying potential candidates.

Results

Several gaps have been identified regarding patients' representation in the network, as the areas of Sickle cell disease (SCD) and Pyruvate Kinase Deficiency (PKD). Finally, considering the large number of diseases included in the lymphoid subnetwork, it has been chosen to having another ePAG supporting the ERN.

- Natacha Bolaños from Lymphoma Coalition has been named ePAGs for Lymphoid Subnetwork.
- The cooperation with a patient representative from PKD Contactgroup has been strengthened and proposed to be formally recognized as ERN-EuroBloodNet ePAG.
- For SCD, more efforts need to be done for identifying an adequate candidate. Description of the ongoing procedure is developed in the paragraph 2.3 of this Deliverable.

The ERN-EuroBloodNet is represented by 9 ePAGs affiliated to 11 different International or European Patients Networks and to EURORDIS too, as it covers all Rare diseases, finally 5 ePAGs contribute also to National Support Groups, as listed:

1. Jan Geissler from CML Advocates Network (International) and Leukemia Patient Advocates Foundation (European)
2. Loris Brunetta from Thalassemia International Federation (International) and Associazione Ligure Talassemici Onlus (National)
3. Sophie Wintrich from MDS UK Patient Support Group (National) and MDS Alliance (European)
4. Baiba Ziemele from Latvia Hemophilia Society (National), European Haemophilia Consortium (European) and Latvian Alliance of Rare Diseases (European)
5. Ananda Plate from Myeloma Patients Europe (European)
6. Maria Piggin from PNH support Group (National) and PNH Global Alliance (International)
7. Pierre Aumont from Ensemble Leucémie Lymphomes Espoir (National) and Chronic Lymphocytic Leukemia Advocates Network (European)
8. Dag Erling Stakvik from European Federation of Associations of Patients with Haemochromatosis (European)
9. Natacha Bolaños from Lymphoma Coalition (International)

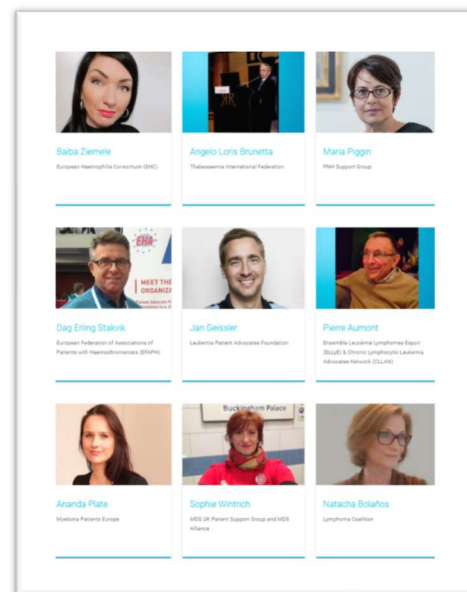


Fig 1. In the figure are listed the Pan-European Network of Hematology Patients Organisations of the 9 ePAGs affiliated to the ERN-EuroBloodNet. All ePAGs are affiliated also to EURORDIS, some of them contributes also to National Support Groups.

Next steps

The designation of an ePAG for PKD and for SCD will be finalized.

2.2 PATIENTS' ADVOCACY ACTIONS ON ERN-EUROBLOODNET'S WEBSITE & PATIENTS ORGANIZATIONS SUPPORTING GROUPS' PROFILE

Rationale

The ERN- EuroBloodNet is coordinating several actions to improve the lives of people living with rare hematological diseases. For each of those actions the Network count on the collaboration of ePAGs or other patients' representatives belonging to local, national, european or international organisations.

Objective

The ERN-EuroBloodNet fixed the objective of raising the visibility of those actions and to patients associations that are actively contributing to the achievement of the ERN's goals. So, major aim was to list and describe each ongoing project and to list and profile the Patients organizations supporting groups. Secondarily, the objective was to underline and recognize the added values of patients representatives collaborations.

Methods

- Creation of a section on the website dedicated to patients' advocacy actions
- Creation of clickable profile for each patients organizations supporting groups

Results

The sections on Patients' advocacy actions has been created on the ERN-EuroBloodNet Website. The bar appears on the main menu and contains 3 items: ePAG Advocates, European Organisations, Patients organizations ERN actions:

ePAG Advocates Sections: It provides the information on the concept of the ePAGs and their missions for the ERNs. In addition, in this sections it is listed who are the ePAGs collaborating with the ERN-EuroBloodNet, the clinical subnetwork they covered and their affiliation to patients' organizations.

European Organizations: In this section, there are listed eleven pan-European umbrella networks that cover a wide range of hematological diseases. Their membership (hematology patient advocacy organisations with members in Europe) comprises of more than 250 national and regional patient organisations that support patients and carers, engage in health policy and engage in research.

There is also the list of three patient organisations are not yet affiliated or represented by a pan-European or international umbrella organisation, but are providing crucial support to patients on the national level. They may even be of help to find similar patient organisations in the same disease area in other countries, in order to provide to RHD patients community a more exhaustive list. Those organisations are listed and by clicking them, the web user can be addressed to the official organisation's webpage.

Patients organizations ERN actions: In this section, the website's user can find the list of the finalized or ongoing actions that are patients-centred and co-coordinated with patients' organizations supporting groups. This list is structured in several boxes (one per project). Those boxes are clickable and in each of them, the user can find the description of the projects, the outcomes achieved (if the action is finalized) and the list of patients' organizations that collaborated to the action. Those organisations are listed and by clicking them the web user can be addressed to the official organisation's webpage.

On the other hand, ERN-EuroBloodNet website back office has been technically upgraded to allow the creation of profiles for **Patients Organizations Supporting Groups** including the Country, geographical coverage, diseases groups covered by the organization (to be selected from RHD disease groups), Umbrella linked to the organization and links. The design ensures the same structure as for the creation of ERN-EuroBloodNet Members profiles, including the selection of the diseases of expertise based on ORPHA classification, therefore ensuring an interlink with all the existing profiles with ORPHA classification as common codification element.

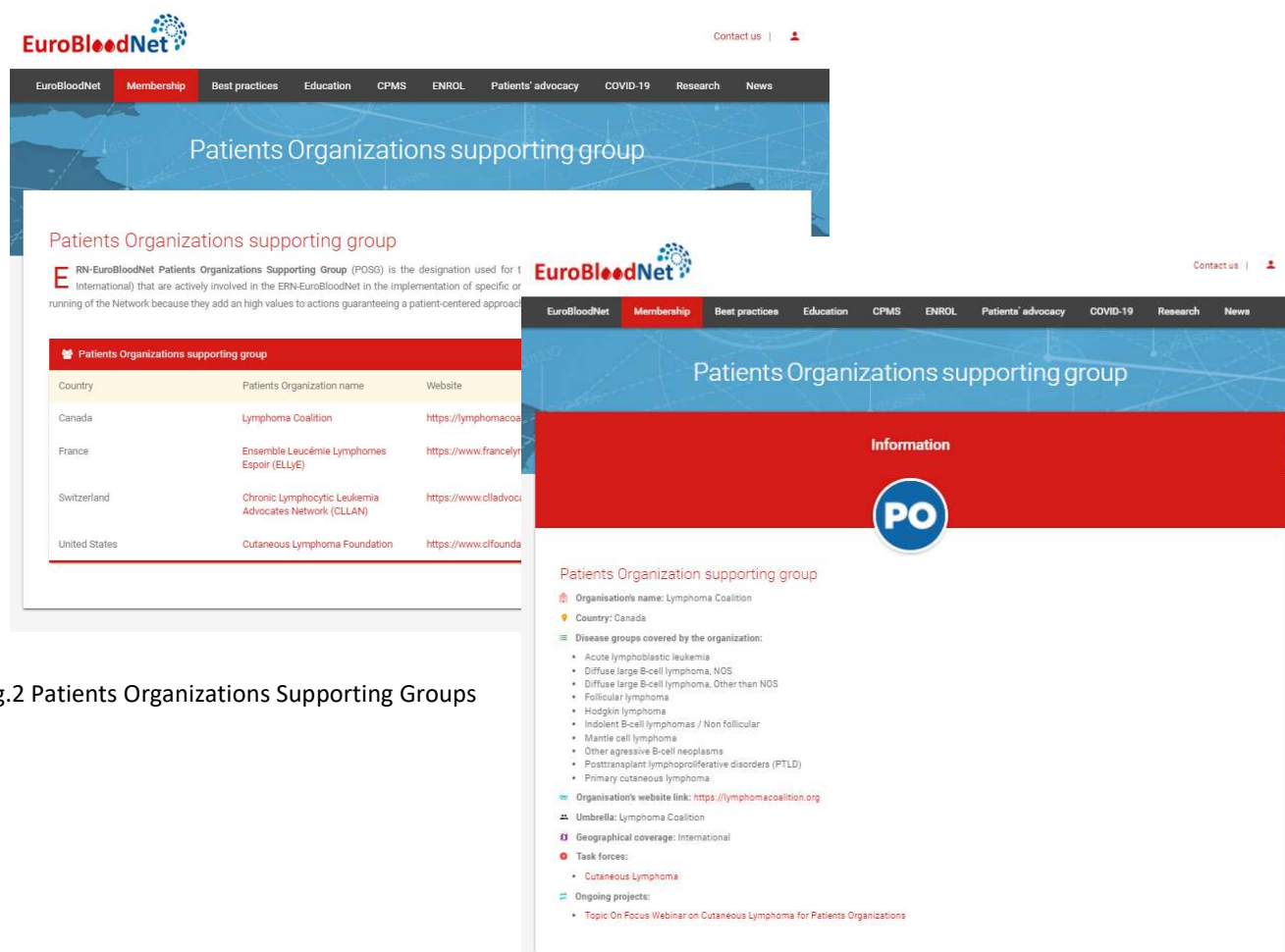


Fig.2 Patients Organizations Supporting Groups

Next steps

European Organizations sections:

- Completing the list patients associations based on the implementation of patients associations profiles at the website
- Adding the following Patients Association Search Tool to the ERN-EuroBloodNet section of European Organizations
 - Orphanet Research Tool for Patients Organizations (available in Italian, French, Spanish, Portuguese, German, Dutch and Polish languages) <https://www.orpha.net/consor/cgi-bin/SupportGroup.php?lng=en>
 - European Haematological Associations Partner Patients associations <https://ehaweb.org/organization/partners/patient-organizations/>

Patients organizations ERN actions:

- Continuously update the section with the outcomes of ongoing and achieved actions

ERN-EuroBloodNet Patients Organisations Supporting Groups:

- The Diseases on Focus Area will be implemented providing information on patients associations who actively contributed to the Network.
 - Patients associations providing collaboration for: Sickle Cell Disease, Aplastic Anemia and PNH will be implemented.

2.3 FACILITATING THE CREATION OF A BOTTOM UP EUROPEAN NETWORK OF SCD PATIENTS ORGANIZATIONS

This action has started in 2017 and its steps and interim results has been presented in the "Deliverable 6.4 Patients Action" of September 2020 and in the deliverable 6.2 Report on promotion of CME on RHD of February 2021. This paragraph is an update of the ongoing action, so past results will not be presented again in this deliverable.

Rationale

Sickle Cell 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. SCD does not have yet in every country in Europe adequate services for patients' daily management of the disease, such as experts centers or patients organizations. As consequence, in Europe SCD patients and their families face different burdens: isolation, no advocacy for SCD patients' rights, no accessible information, no psychological and human support and no connection with expert centers. Therefore, 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.

The ERN-EuroBloodNet SCD Task Force, which is formed by Coordination Team, ePAGs Loris Brunetta (representatives of Red Blood Cell defects for and patient advocate for Thalassemia, the most similar disease to SCD) and SCD expert members of the Network identified in 2017 the urgent need of having a solid representation of SCD in Europe. As said, in many European Countries there are no patients association covering SCD. Therefore, the task force have analyzed together with EURORDIS how to cover the lack of patients associations identified in most of the European Countries, and developed the comprehensive strategy since 2017 for facilitating the establishment of a a patient-driven European network of SCD patient organisations . That is to say, a group of SCD patients advocates coming from European Countries that once contacted by ERN-EuroBloodNet and Eurordis, would decide to create a European Network of SCD Patients Organizations (POs).

The ERN-EuroBloodNet and EURORDIS over the past years, and starting from 2017, have worked towards the aim of facilitating the establishment of a patient-driven European network of SCD patient organisations. National meetings with local SCD Patient Organisations have been organized in Belgium, France, Italy, Spain & Portugal to bring together the local patient organisations and raise awareness of the activities of the ERN-EuroBloodNet and EURORDIS. In the UK in 2019, within ASCAT Steering Committee (Annual Congress on Sickle Cell and Thalassemia), a first Educational session organised with the ERN-EuroBloodNet took place to discuss with SCD patient representatives the "Top 10" priorities on research from their perspective. A second edition took place online in 2020 and another one in January 2022 and a new one will come in October 2022. (Those outcomes are presented in the paragraph 4.1 of this deliverable)

Interim results reached so far were: (those results have been presented in the Deliverable 6.4 on Patients Actions of September 2020 and on the Deliverable 6.2 Report on promotion of CME on RHD of February 2021)

- More than 50 patient organisations involved in Europe and 10 out of Europe in actions carried by the ERN-EuroBloodNet.
- National patients' representatives identified for Italy, France, Spain, Portugal and Belgium Therefore, EURORDIS and ERN-EuroBloodNet planned the next step to overtake. That is to say organizing a first meeting with all the patients associations reached during those year for evaluate the possibility of facilitating the creation of a bottom up European Network of SCD Patients Organizations.

Objective

The ERN-EuroBloodNet decided to have a kick of meeting with all the SCD Patients representative supporting the ERN, in order to start training them at advocacy at the European Level and secondarily to identify among them potential candidate for being SCD ePAGs.

Specifically to the step to be overtaken to achieve this ambitious objective, it has been fixed the objective to launch a first meeting of the identified SCD patients' organizations in Europe and elected representative in order to:

1. Evaluating the maturity of the plan of facilitating the creation of a bottom up European Network of SCD Patients Organizations
2. Evaluate along the years the consistency of motivation and interested of contacted patients organizations
3. Start identifying first potential actions carried by the POs Group, the ERN and EURORDIS
4. Identifying first potential SCD candidates for becoming ePAGs

Methods

EURORDIS, ePAG for Red Blood Cell Defects Loris Brunetta and ERN-EuroBloodNet defined the structure to prepare the meeting, including the agenda and selecting the key messages to ensure discussion with participants collecting feedbacks and answering questions on the establishment of a roadmap of priority actions to carry out collectively

Invitations were sent to the 50 SCD patients association reached in Europe and constantly in contact with the ERN-EuroBloodNet for participating to the ongoing actions dedicated to SCD, Patients Associations and patients motivated to contribute to the cause of SCD advocacy in Europe in contact with the Coordination Team of the ERN-EuroBloodNet and Patients' representatives already elected for being part of the European Network of SCD Patients' Organisations (Italy, France, Belgium, Spain and Portugal).

Results

32 people covering 13 European Countries: Spain, Portugal, Italy, France (France metropolitan and France Overseas), Cyprus, Belgium, Greece, Ireland, UK, Netherlands, Switzerland, Sweden, Denmark, registered for the meeting

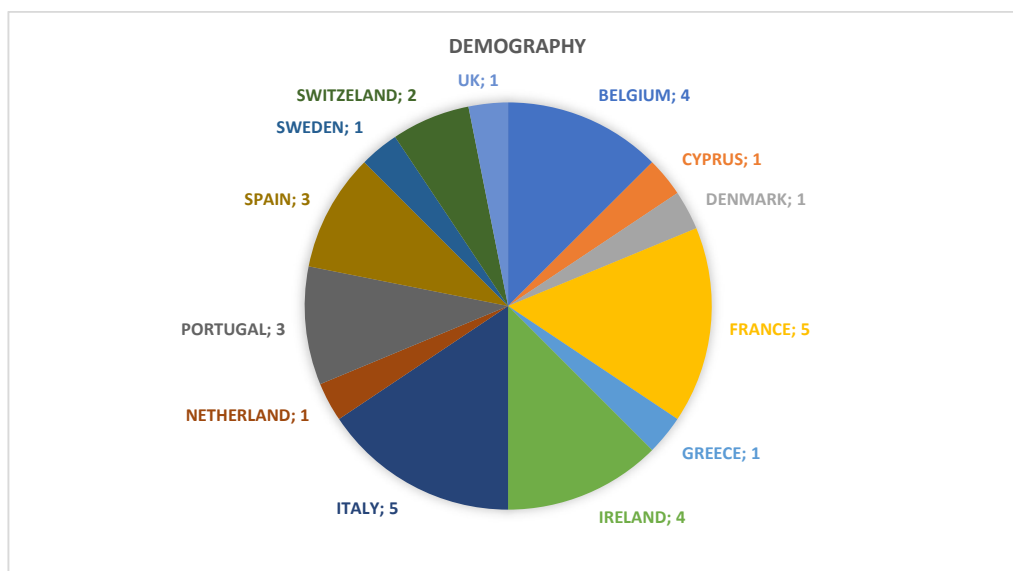


Fig.3. demography of participants registered to the meeting

The following points, in line with the meetings held between France, Italy, Spain, Portugal and Belgium are common points raised and could be translated into first concrete advocacy actions at European level: Recognition of Sickle Cell Disease as recognized disability in the National Table of Disabilities, fight against Stigmatization and patient isolation, improving best practices and patients' pathways, awareness raising on the importance of receiving psychological help, neonatal and prenatal screening programs and improving the visibility of the disease during the International Sickle Cell Disease Awareness day.

Next steps

- Organization of a Survey for prioritizing the first actions that the informal group could carry on with the ERN.
- Envisaging a next meeting for keeping the group together and shaping the actions based on survey results.
- Continuing the work of identifying potential candidate for becoming ePAGs.

3. EUROPEAN CROSS BORDER HEALTH ASSISTANCE TO PATIENTS

EUROBLOODNET CROSS BORDER HEALTH ASSISTANCE TO RARE HEMATOLOGICAL DISEASES PATIENTS, AN INFOPOINT FOR PATIENTS DEALING WITH CROSS BORDER HEALTH RIGHTS

Rational

Directive 2011/24/EU of the European Parliament and of the Council on the application of patient rights in cross-border healthcare provides rules regarding access and reimbursement for healthcare received in another EU country in order to encourage cooperation between EU Member States in the field of health. With the creation of the European Reference Networks (ERNs), the EU has provided the requirement and the means for providing cross-border health assistance to RHD Patients, as ERNs are structure for knowledge sharing and coordination of health care across the EU.

One of the ERN-EuroBloodNet most ambitious goal is the establishing of a model for cross border referral system for patients and samples based on patients' pathways implementation and in accordance with Directive 2011/24/EU. For achieving this objective, it is important to monitor and report patient-experienced challenges on cross-border related issues and providing input for improving the referral system.

Objective

The ERN-EuroBloodNet could play a key role in assisting RHD patients that want to benefit from a temporary cross-border health right in accessing healthcare services abroad according to the application of the Regulations (EC) 883/2004 and 987/2009 and the Directive 2011/EU/24. Principal role of the ERN-EuroBloodNet is to help patients navigating the healthcare social systems in Europe by offering a mediation among National Contact Points for Cross-Border Care and hospital administration. Secondly the request of cross border health assistance could provide study cases for the analysis of the Directive 2011/24/EU and the establishment of a referral system.

Methods

The ERN-EuroBloodNet has received requests of Cross Border Health assistance directly from patients who found contact information on the ERN-EuroBloodNet website, or via patients associations that addressed the patients to the ERN-EuroBloodNet Coordination Team.

All the requests and the process of solving the request has been gathered in a repository.

ERN-EuroBloodNet assistance to patients comprises also searching for Clinical expertise abroad and support accessing Cost Effective Treatments. Those topics are not directly included in the basket of benefit of the Directive 2011/24/EU nor the Social Security Regulation (EC) 883/2004 and 987/2009, but it implies a cross-border collaboration among HCPs, health systems, patients and their families.

Therefore, the ERN-EuroBloodnet deals with four different types of patients' assistance:

- Continuity of cares moving abroad in a European Countries
- Dealing with Cross-border Health Rights&Procedures
- Searching for Clinical expertise abroad
- Accessing Cost Effective Treatments or Clinical Trials

Often, one case match with more than one type of assistance, ie. Continuity of care moving abroad and looking for clinical expertise abroad, searching for Clinical expertise abroad and dealing with Cross-border Health Rights&Procedures.

A repository of the requests for assistance received by the coordination team from the beginning of the network has been created, including the main needs for each case and steps followed until the assistance provided to the patients.

Results

Since the launching of the ERNs, the ERN-EuroBloodNet has received 24 requests of cross border health assistance:

- 16 successful
- 4 procedures stopped by patients themselves:
 - Additional information where requested to patients and not provided
 - Covid-19 Pandemic Outbreak blocked the possibility of going abroad
- 2 ongoing
- 2 unsuccessful

First cases have been analysed in the Deliverable 6.4 Patients Actions of September 2020 and Deliverable 4.6 ERN-EuroBloodNet: a model for cross border referral system of June 2021.

The ERN-EuroBloodNet assisting those 24 cases has:

- Provided information about Cross-border Health Rights
- Provided Patient Therapeutic Educational Material thanks to cross border exchange among patients associations
- Searched for expert centers/expert physicians or patient association via the Network
- Moderated among cross-border health actors: National Contact Points, HCP Administrations, Social Security Funds
- Supported access to treatments for rare diseases (e.g providing clinical experts' opinion, writing letters of support to health authorities based on evidence of treatments benefits)

Here it goes the list of the cases received:

1. 2018. Beta Thalassaemia patient moving residence from Italy to France and wanted to access to reimbursed transfusion. Not a reason of cross-border health assistance. Obstacles: passing from one social security to another require time in which the patient had complications in accessing transfusion. Solution: SCD patients found a job and automatically obtained the social security assistance.
2. 2019: a Beta Thalassaemia patients moving from Italy to France for a short-term job contract. Patient wanted to access to reimbursed transfusions. Not a reason for cross-border health assistance. Obstacle: passing from one national social security assistance to another require time in which the patient has complications in accessing healthcare services. Solution: the SCD patients helped with local administration.
3. 2019. Successful example of cross-border health Assistance for Chronic conditions and programmed cares. Italian student in Erasmus study program in France had to schedule cares for thalassaemia. ERN contacted the French NCP for cross-border health and asked mediation among patient' home and host countries social security funds. Patient benefit from the temporary cross-border mobility.
4. 2019. SCD patient (20 years, France) studying in Madrid had burdens in receiving transfusion for lack of knowledge. ERN gave the availability to contact expert physicians in Spain but the patient did not answered back.
5. 2019. Request for finding expert centers in Scotland that could follow the patients' management of three SCD pediatric patients moving from France. Not cross-border health Case. But the ERN identified HCPs and physicians in Scotland.
6. 2019. Request for finding SCD expert center in Spain for an adult patient moving there. Not cross-border health Case. But the ERN identified expert center.
7. 2020. Request from an Italian Thalassaemia patient for finding an expert center in UK. Request stopped with the outbreak of the covid 19 pandemic.
8. 2020. PNH Adult Patient from Bosnia (not EU MS) needed to access to Clinical Trial. ERN support has been provided by PNH expert member offering to edit an official request for access to treatment in patient own country (Bosnia).
9. Four different cases have been registered respectively one in 2019 and three in 2021 for accessing a cost-effective treatment for PNH in their own country: Belgium, Lithuania, Poland and Malta. These requests have been solicited by ERN-EuroBloodNet ePAGs for Aplastic Anemia and PNH. The action has required a coordinated plan among European Network of Patients Association lead by an ePAGs, PNH experts members of the Network and ERN-EuroBloodNet coordination team. For the case of Belgium, Lithuania and Malta, ERN-EuroBloodNet PNH experts stated in official clinical document that that treatment is crucial for patient life, after receiving clinical records. For Poland the request was not linked to one specific patient, so EBN PNH experts and PNH ePAG prepared letter to Health Authorities recommended the access to the treatment as scientific evidence of its efficacy. For Belgium case, the patient got the access to treatment. For Lithuania and Belgium the reimbursement of the treatment has been accepted by health authorities. For Poland the access to treatment has been denied by health authority. Malta's case is ongoing.
10. 2021 for an Italian adult patient affected by Anemia Fanconi asking for an expert consultation of Bone marrow transplant side effects in France where he received a transplant of bone marrow when he was a child. Patient will be accompanied in Cross-border Health related procedure for obtaining the reimbursement of the expert visit. Patient stopped the procedure.
11. 2021 for a second opinion on a case of Composite Lymphoma. Expert was identified at national level but the patient stopped the procedure.
12. 2021 for a SCD adult patient participating in an ERASMUS project in Germany. Request was arrived via the French National Network of Rare Blood Cell Disorders. The ERN found the expert and inform the patient that cares are in the in light of European legislation (Regulations (EC) 883/2004 and 987/2009), patient is considered to be temporarily residing in Germany during his/her studies.
13. 2021and 2022 Five cases for SCD adult patients searching for clinical expertise abroad because of moving in another country. From France to Germany, from France to Hungary, from Ireland to Germany, from France to Romania, from Spain to

Malta. Request arrived from Affiliated Partner and from Patient Associations. Expertise was found among ERN members and supporting partners.

14. 2021 for a second opinion on a case of deficiency of the factor XII (Hageman factor) requested by a caregiver (mum) for a teenager patient. Expertise was found at national level.

15. 2021 for an adult patient affected by lymphomatoid papulosis searching for patient therapeutic educational material on this disease. Contact with patients associations has been provided and material furnished. The ERN has been contacted because of the visibility given by a webinar on another rare lymphoma addressed to patient.

16. 2021 for a second opinion on Cutaneous Lymphoma on aging patient management. The ERN has been contacted because of the visibility given by webinar programs addressed to Cutaneous Lymphoma. Patient was put in contact with two experts of the ERN-EuroBloodNet who provided with the second opinion.

17. 2021 Beta Thalassemia Italian adult patient looking for an expert center in Portugal. The ERN was contacted via the ePAG on Rare Blood Cell Disorders and expertise found via ERN-EuroBloodNet expert members.

Analysis

From the requests received, the following important outcomes could be considered:

1. ERN are a solid support for Cross-border Health Assistance to patients and could play a pivotal role in mediation with administration, National Contact Point, patients and physicians. More specifically the role of the ERN-EuroBloodNet has been:
 - a. Providing information about Cross-border Health Rights.
 - b. Searching for expertise via the Networks: Members, Affiliated partners and candidates and giving information about cross-border health if needed
 - c. Moderators among cross-border health actors: National Contact Point, HCPs, Patients
 - d. Supports access to treatment e.g providing clinical expert opinions, writing letters of support
 - e. Provided Patient Therapeutic Educational Material thanks to cross border exchange among patients associations
2. No recognitions of the Directive 2011/24/EU or Regulations (EC) 883/2004 and 987/2009 in hospital administration and at National Level (social security).
3. Transfer from one country to another could be very problematic for patients suffering from chronic conditions and requesting programmed care. The procedure of moving from one national social security assistance to another are time consuming. This is not an aspect foreseen in the framework of Directive 2011/24/EU or Regulations (EC) 883/2004 and 987/2009, but crucial point that could be addressed by European Member State Social Regulations.
4. For many different rare haematological conditions, programmed cares as transfusions need to be performed. In many countries, accessing to those programmed for a patients coming from another European country in the framework of the Directive 2011/24/EU or the Regulations (EC) 883/2004 and 987/2009 is full of administrative obstacles at local and national level. In addition, Information sometimes are not clear and the patients feel lost in procedures.

Next steps

It has been decided to adding a "Contact us" section on the ERN-EuroBloodNet website for assisting Cross Border Health requests will be evaluated.

4. EDUCATIONAL ACTIONS IMPLEMENTED

One of the ERN's most ambitious objectives is the promotion of education for patients targeting rare diseases. It is very difficult to produce and/or find accessible educational material because of the low prevalence of rare diseases themselves, the lack of patients associations that serve as an info point, the lack of expert centres that could help patients in receiving information. This situation directly affects the possibility for rare disease' patients to receive an adequate education with respect to the pathology they suffer from. The educational opportunities for a patient interested in therapeutical education are not many nor easily accessible, mostly if the condition belongs to non-oncological hematology's clinical domain, or to ultra-rare conditions. In addition, the organisation of patients training is 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). Only if an experienced patient association covers a disease or a group of diseases there is the possibility to find highly quality validated educational documents and get the patient empowered.

This is the reason why the ERN-EuroBloodNet is fruitfully investing on educational projects addressed to both health professional and patients. As stated in the deliverables: "Deliverable 5.1 Report of educational gaps" of 2018, " 6.1 ERN-EuroBloodNet Report on Actions for the promotion of continuing medical education on RHD", "6.4 Patients actions" of 2020 and "6.2 Report on Actions for the promotion of Continuing medical education on RHD" of 2021 in the previous period of network implementation.

Education is the major basis for increasing patients' empowerment, and it should not just have an informative role, like a mere transmission of knowledge, but it should include a comprehensive pedagogical plan. The plan should include giving visibility and representation of rare disease in Europe, promoting advocacy training and educational training, creating educational material repositories, encouraging the establishment of patients associations, strengthen the collaboration between physicians and patients, etc. This is the reasons why the ERN-EuroBloodNet is coordinating different joint project for reaching the objective of improving rare disease patients' empowerment, such as:

1. On-site and Online Educational Patients Training (included mental health awareness and patient therapeutic education)
2. Repository of Educational Material for patients
3. Comprehensive webinar program for patients and their families or patients associations
4. Patients Testimony videos & International awareness' campaign

In this section, the ERN-EuroBloodNet' educational actions are described, both past projects and ongoing projects that are specifically addressed to patients, caregivers, patients advocates and patients organizations

4.1 SICKLE CELL DISEASE

4.1.1 EDUCATIONAL PATIENTS SESSION AT CONGRESS: SCD PATIENTS EDUCATIONAL SESSION AT ASCAT 2020 AND ASCAT 2022 JANUARY

Rationale

ERN-EuroBloodNet is promoting educational activities taking place during International Scientific Congress and targeting people living with SCD since 2019, in order to encourage their training and develop their skills as advocate and expert patients. First reason for coordinating this project is that when it comes to SCD in Europe is frequent to face patients' isolation. Indeed, there are few existing SCD patients' associations and most of the patients do not have an adequate educational level as they come from third countries and suffer from immigration burdens. Therefore, the ERN-EuroBloodNet considers those events as an occasion for SCD patients to meet each other in a dedicated space for them and sharing experience and expectations.

In addition, the ERN-EuroBloodNet's model of educational sessions at International Scientific Congress is a way to make visible to scientific community what patients would prioritize as research topics and tackle gaps to address. Finally, this experience strength also the cooperation among health professionals, patients and caregivers, because they learn to listen to each other and to share ideas, needs, and possible projects to build together. Indeed, as explained in next following paragraphs, SCD patients could present the outcomes of the educational sessions they took part to, at the plenary session of International Scientific Congress.

The outcomes of the 2019 session, named "Sickle Cell Disease Research Prioritization Workshop at ASCAT 2019" have been presented in the "Deliverable 6.4 Patients Action of September 2020".

Objective

The SCD Sickle Cell Disease Patients Educational Sessions at the Annual Scientific Conference on Sickle Cell and Thalassaemia (ASCAT) is joint project of the ERN-EuroBloodNet together with ASCAT members aiming to:

- Creating a group of people living with Sickle Cell Disease (European and International) for let them expressing their needs and share experience and best practices
- Train patients to Patient Therapeutic Education and Public Patient Involvement in Research
- To evaluate in Europe the impacts of Patient Therapeutic Educational session on patients and their families
- To create further connections among health professionals and patients
- Making visible to scientific community patients' needs and expectations

SICKLE CELL DISEASE PATIENTS EDUCATIONAL SESSION AT ASCAT 2020

Considering the success of the SCD patients' session at ASCAT 2019, it was decided to repeat the experience by organizing another educational event addressed to SCD patients in the framework of the **15th Annual Sickle Cell and Thalassaemia Conference (ASCAT)** <https://web-eur.cvent.com/event/4c632bd1-0e87-4d69-a2b3-8d3bce1b08e4/summary>. ASCAT 2020 Conference has been organized in collaboration with the European Hematology Association (EHA) and the British Society of Haematology (BSH), from the 26th to 31st of October, on an online virtual platform as a result of Covid19 Pandemic Outbreak.

A part letting SCD patients participating to the Educational Online session specifically addressed to them, the ERN-EuroBloodNet Coordination Team and the ASCAT members also gave the possibility to assist to the whole ASCAT Congress event. This was an important opportunity for SCD patients and parents patients to learn more and discuss the latest advances in terms of diagnosis, treatment and emerging new therapies in the field of the haemoglobinopathies as the ASCAT 2020 focused on 'Haemoglobinopathies: Emerging Challenges and Future Therapies'.

Methods

Together with ASCAT team, EuroBloodNet Association planned for SCD patients an online Educative Session, programmed on three different days. A team of SCD experts belonging to ERN-EuroBloodNet and ASCAT committee led the educational session. Prof. Beatrice Gulbis, from Hôpital Erasme/LHUB-ULB in Belgium; Prof Mariane de Montalembert, from AP-HP hôpital Necker, in France; Dr. Noemy Roy from Oxford University Hospitals NHS Foundation Trust, Dr Baba Inusa from Guy's and St Thomas' NHS Foundation Trust, Dr Raffaella Colombatti, from AO Padua in Italy and finally CHAKRAVORTY, Subarna, from King's college Hospital NHS Foundation Trust. Also EURORDIS represented by Ariane Weinman and ePAGs Loris Brunetta are contributing to the development of the SCD patient session program.

The above-mentioned group together with ERN-EuroBloodNet coordination team decided the topics to raise during the online educational session this year. The SCD Patients Educational Session involves three major topics:

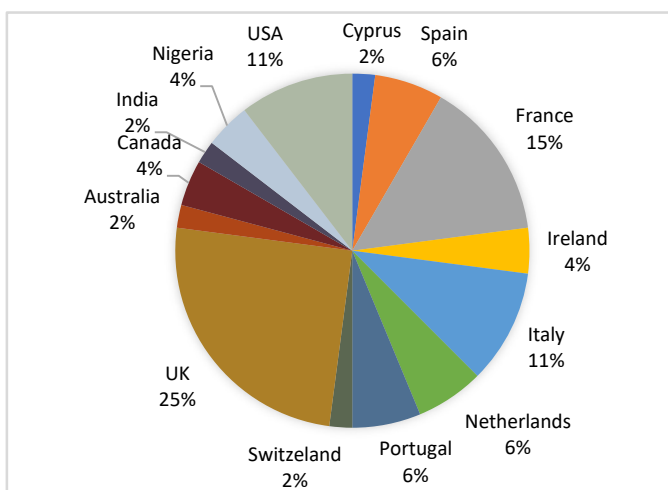
- Living with SCD and coping with COVID19 Pandemic Outbreak, (access to hospitals, management of crisis, receiving information about Covid, etc.)
- The participations of SCD patients to Research and Peer Reviewing.
- Three panels. One "meet the experts" and two "meet the patients' session". There will be the possibility for patients to raise dedicated questions to physicians and for physicians and young patients, the possibility to raise questions to adult patients.
 - For the panel "meet the experts" five topics were identified via a patient surveys called "ERN-EuroBloodNet's Survey for prioritizing SCD topics to be addressed by Education" that has gathered 24 answers expressed by SCD patients coming from: France, Italy, Spain, Portugal, Cyprus, USA, Belgium. Topics were; newborn and infant SCD screening, neurological complications in SCD adult people, quality of life in SCD, survival, infertility and other complications of bone marrow transplantation and new therapies for SCD. This survey has been presented in the Deliverable 6.4 Patients Actions of June 2020.
- Living with SCD as pediatric or young adult patients.

As visible in the "Annex I ASCAT 2020", presenting the agenda of the ASCAT 2020 patients' session (together with final outcomes and patients feedback survey), a duo made by physician and patient representative moderated each topic session. Their role was presenting the topic, gathering key messages and collecting questions raised by hearers in the platform's chat. For some topics, pre-recorded session made by patients' testimonies was shared. For better shaping the framework of each specific session, the ERN-EuroBloodNet Coordination Team has collected questions and key messages before the official day of the Educational SCD session at ASCAT.

Results

68 participants registered for the online event: 48 patients and parents and 20 health professionals or other role, mostly from European countries.

Fig. 4 Distribution of the 48 participants (SCD patients and parents patients) by country of living participating at SCD Patients Educational Session at ASCAT 2020



- The Educational Online Session was fully recorded, participant signed an informed consent for sharing the recordings, in line with the General Data Protection Regulation (GDPR). Those videos are available on the YouTube Educational channel, EuroBloodNet Edu's and divided into 2 different playlists:
 - a. [\(13 videos\) Patient's Testimony. SCD Patients' Educational Session at ASCAT 2020](#)
 - b. [\(9 videos\) SCD Patients' Educational Session at ASCAT 2020](#)
- The Feedback Survey to the SCD Educational Session participants in order to understand how to improve the program in upcoming year scored as excellent. 93% of participants rated with 4 or 5 out of 5 in overall evaluation for the event, 89% gave high scores on the topics discussed, >90% approved the patient and clinician jointly moderated sessions with high ratings and 95% preferred the online format of the program. For the 2020 session, the COVID19 pandemic resulted in the conversion of the conference to an online one, allowing a much wider participation by patients and careers who would otherwise not have been able to travel to Central London due to work, health or childcare issues.
- Four new educational needs were identified during the SCD Educational session: racism; mental health, pregnancy, and side effects of bone marrow transplantation. Those topics have been addressed during ASCAT 2022 (January)
- Patients group required a Webinar dedicated to COVID 19. As described in “Patients Webinars” next section of this deliverable, it has been conducted the 14th of December 2020. Results are also in the mentioned paragraph.

SICKLE CELL DISEASE PATIENTS EDUCATIONAL SESSION AT ASCAT 2022 (JANUARY)

Rationale

ERN-EuroBloodNet is promoting educational activities taking place during International Scientific Congress and targeting people living with SCD since 2019, in order to encourage their training and develop their skills as advocate and expert patients. First reason for coordinating this project is that when it comes to SCD in Europe is frequent to face patients' isolation. Indeed, there are few existing SCD patients' associations and most of the patients do not have an adequate educational level as they come from third countries and suffer from immigration burdens. Therefore, the ERN-EuroBloodNet considers those events as an occasion for SCD patients to meet each other in a dedicated space for them and sharing experience and expectations. In addition, the ERN-EuroBloodNet's model of educational sessions at International Scientific Congress is a way to make visible to scientific community what patients would prioritize as research topics and tackle gaps to address.

Finally, this experience strength also the cooperation among health professionals, patients and caregivers, because they learn to listen to each other and to share ideas, needs, and possible projects to build together. Indeed, as explained in next following paragraphs, SCD patients could present the outcomes of the educational sessions they took part to, at the plenary session of International Scientific Congress.

The outcomes of the 2019 session, named “Sickle Cell Disease Research Prioritization Workshop at ASCAT 2019 and 2020” have been presented in the “Deliverable 6.4 Patients Action of June 2020” and Deliverable 6.2 report on promotion of CME on RHD of September 2021.

Objective

The SCD Sickle Cell Disease Patients Educational Sessions at the Annual Scientific Conference on Sickle Cell and Thalassaemia (ASCAT) is joint project of the ERN-EuroBloodNet together with ASCAT members aiming to:

- Creating a group of people living with Sickle Cell Disease (European and International) for let them expressing their needs and share experience and best practices
- Train patients to Patient Therapeutic Education and Public Patient Involvement in Research
- To evaluate in Europe the impacts of Patient Therapeutic Educational session on patients and their families
- To create further connections among health professionals and patients
- Making visible to scientific community patients' needs and expectations

Also for the 2021 the collaboration with The Annual Scientific Conference on Sickle Cell and Thalassemia and the ERN-EuroBloodNet for organizing the patients educational session was confirmed. By the way, due to Covid-19 outbreak, it has been decided by ASCAT Steering Committee to move the conference from October 2021 to January 2022 (from 26th to 28th of January) and to hold it online. It is important to keep the representation of patients during this International Conference because this is one of the must attend events of the year for consultants and specialist psychologists, nurses, scientists and all relevant experts, so it is crucial that this event have patients representation at its core. Patients and partents patients invited to the Educational session have also the opportunity to participate to the full ASCAT congress and learn about the latest advances in diagnosis, treatment and emerging fields in haemoglobinopathies, and build a solid dialogue among patients and health professionals. ASCAT 2022 January has been organized in collaboration with European Hematology Association & British Society for Haematology, and was focused on the topic of ‘Improving the lives of people living with Sickle Cell Disease and Thalassaemia: a focus on new therapies and person-centered care.’

For the ASCAT 2022 it has been decided to open the patients' educational sessions to the all participants of the ASCAT congress. So the session was advertised in the general program, and audience could access not only via individual invitation to the Educational session, but also via general ASCAT 2022 platform. Strategy chosen for the first 2 editions, because the forum of patient was still not mature enough and it could be a lack of opportunities for participants to openly express themselves.

Methods

Together with ASCAT team, EuroBloodNet Association planned for SCD patients an online Educative Session, programmed on three different days, from the 26th to the 28th of January. The agenda is in the Annex II: ASCAT 2022 January

A team of SCD experts belonging to ERN-EuroBloodNet and ASCAT committee led the educational session. Prof. Beatrice Gulbis, from Hôpital Erasme/LHUB-ULB in Belgium; Prof Mariane de Montalembert, from AP-HP hôpital Necker, in France; Dr. Noemy Roy from Oxford University Hospitals NHS Foundation Trust, Dr Baba Inusa from Guy's and St Thomas' NHS Foundation Trust, Dr Raffaella Colombatti, from AO Padua in Italy and finally CHAKRAVORTY, Subarna, from King's college Hospital NHS Foundation Trust. Also EURORDIS represented by Ariane Weinman and ePAGs Loris Brunetta are contributing to the development of the SCD patient session program.

Topics were pre-identified by participants of previous edition: Racism, Mental Health, Pregnancy and Bone Marrow Transplant, so it has been decided to have two interactive webinars and two interactive panel:

- SCD Patients Educational Session by ERN-EuroBloodNet&ASCAT: Spotlight on Racis : an interactive webinarwith patients testimonies lead by expert physicians. Open discussion with the audience.
- SCD Patients Educational Session by ERN-EuroBloodNet&ASCAT: Spotlight on Mental Health : an interactive webinar with patients testimonies and lead by two specialised psychologists. Open discussion with the audience
- SCD Patients Educational Session by ERN-EuroBloodNet&ASCAT : Spotlight on Pregnancy: an interactive panel lead by an expert physician, a specialised obstetrician and a patients representatives. Open discussion with the audience.
- SCD Patients Educational Session by ERN-EuroBloodNet&ASCAT: Spotlight on Bone Marrow Transplant: an interactive panel lead by one expert physician and a patient representative. Open discussion with the audience

Also for this edition, and in line with the general objectives of the project, participants of the sessions presented the outcomes at the closing plenary session of the ASCAT 2022.

Results

54 people registered to the only event: 34 people living with SCD and 20 health professionals, mostly from European Country.

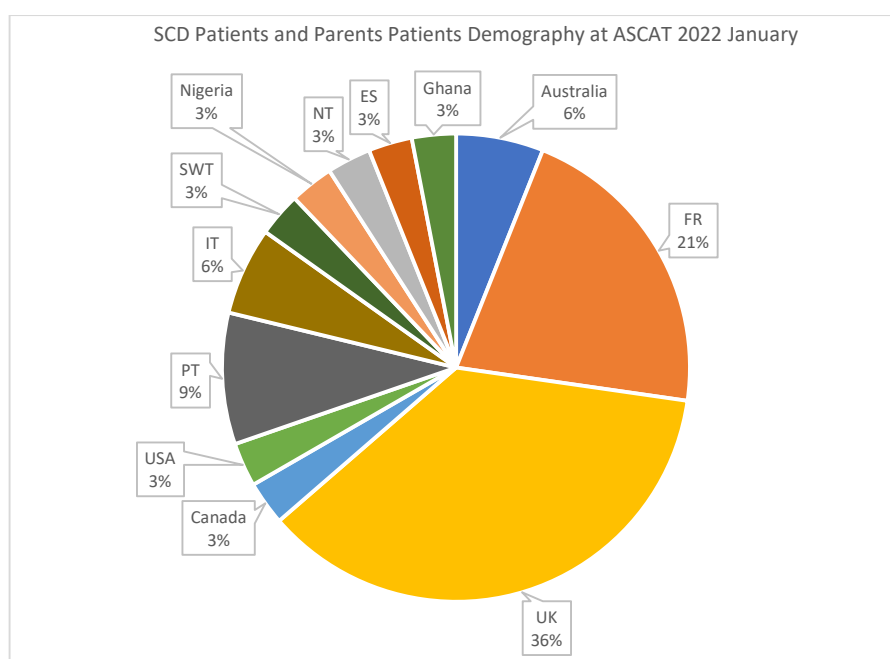


Fig. 5 Distribution of the 48 participants (SCD patients and parents' patients) by country of living participating at SCD Patients Educational Session at ASCAT 2020

- An average of 50 extra people connected to the patients' session through the general ASCAT 2022 congress platform.

- To open the Patients Session also to ASCAT 2022 Participants (health professionals, Consultant Haematologists, Paediatricians, Specialist Nurses, Psychologists, Scientists, Fellows and trainees in Sickle Cell fields) has had good impact on the running of the event. The interaction among health professionals and people living with SCD was very productive manifesting a mutual willing of listening each other.
- Presentation of the outcomes of the SCD Patients Educational Session at the ASCAT 2022 January at the closing plenary session of ASCAT 2022. Presentation is in Annex II: ASCAT 2022 January
- The 4 sessions have been recorded participants signed an informed consent for sharing the recordings, in line with the General Data Protection Regulation (GDPR). Those videos will soon be available on the YouTube Educational channel.

General Conclusions from the experience of three editions:

- Involving SCD patients in research and care is difficult and not often done. This disenfranchised group is typically challenging to engage. With our model of Educational training, we show to patients' community and scientific community that it is possible and meaningful, it makes a difference to the patient group and it strength the relation among physicians and patients. Finally, it has been highly appreciated from scientific and patients community to hear patients perspective during international scientific conferences.
- Patients group required educational trainings and find it increase their quality of life
- Envisaged obstacles have been the different levels of patients' educations and the language fluency, as the whole conference is in English. Whereas the online format has been appreciated by patients. There are no burden for family care of days of work, no fatigue of travelling, patients felt free to raise questions thanks to the comfortable situation for addressing questions in the chat.

Next steps

- Editing and publishing the video of ASCAT 2022 January

4.1.2 ERN-EUROBLOODNET PARTICIPATING TO THE WORLD SICKLE CELL DAY 2020 AND 2021

Rationale

In Europe, people living with SCD mainly belong to ethnic and social minorities. Therefore, they are more isolated, have a lower level of health education, poorer adherence and disease management compared with patients with other hematologic diseases. Those conditions make the disease invisible to the society. The ERN-EuroBloodNet could provide a worldwide visibility to Sickle Cell Disease and correlated patients' needs, by monitoring and reporting patient expectations thanks to the results obtained by the several projects dedicated to the SCD the ERN have launched. The ERN-EuroBloodNet has considered a fundamental action to contribute to the World Sickle Cell Day 2020 by giving voice to SCD patients. Main aim was indeed to train patients in giving testimonies, spread patients' needs and expectations by giving to patients the possibility of expressing themselves and providing editing and recording solutions, media channels and logistics assistance.

SICKLE CELL DISEASE INTERNATIONAL WORLD AWARENESS DAY 2020

Objective

Given visibility to patients' voice by publishing a video on our ERN-EuroBloodNet Educational YouTube Channel is a cardinal action aiming to raising awareness on Sickle Cell Disease at public at large, scientific community and policy makers.

Methods

The ERN- EuroBloodNet has first contacted the SCD patients and parent's patients that have taken part to the 2 Educational events: SCD Research Prioritisation Workshop at ASCAT 2019 and to the National meetings for the establishment of the European Network on SCD Patients Organisations, by sharing the idea of producing a testimony video for the International Day of SCD. To those patients willing to participate, the ERN-EuroBloodNet has given the following instructions:

Video duration should be less than 1 minute

- Language should be English
- Video's testimony could be developed in those suggested frameworks:
- National meetings: "What do you expect from an European common patient's voice?" Or "How did you felt in having the opportunity to share your experience and concerns with other patients?", "How do you imagine will be the opportunity to exchange with patients coming from other EU countries?"
- ASCAT 2019: "What do you expect from Research and/or what do you expect from patients' involvement in Research?" Or "How did you felt in having the opportunity to take part to the SCD Research Prioritization workshop and share your thoughts and needs with patients from other EU countries?".

- Video should be recorded in horizontal
- Consent form signed by each testimony for sharing the video for the International day of the SCD published on ERN-EuroBloodNet website and social channels.

Secondarily, the ERN-EuroBloodNet have done an editing work of video received. Technical support has been provided by ERN-EuroBloodNet CPMS & e-learning Manager, as part of a new Grant by the EC for the CPMS and eLearning platforms that has been officially launched 1st October 2019. The Educational channel launched on YouTube by the ERN-EuroBloodNet is indeed part of the development of the e-learning platform of the ERN-EuroBloodNet.

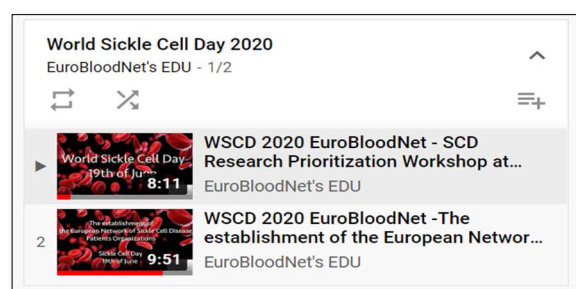


Fig.6 The two videos' testimonies published for the WSCD 2020

1st Testimonies Video, the establishment of the European Network of SCD POs

The ERN-EuroBloodNet together with EURORDIS is establishing the European Network of Sickle Cell Disease Patients' Organisations aiming to provide a centralized European point of contact for patients and caregivers. Secondary objective is to create synergies among existing national patients support groups and finally objective is to encourage the creation of national patients association in those country where patients association do not exist yet.

A total of four National meetings (Italy, France, Spain&Portugal and Belgium) has been held during third and fourth year of the ERN-EuroBloodNet.

For the World Sickle Cell Disease Awareness Day 2020 (WSCD) two SCD patients' testimony videos have been published on the EuroBloodNet's YouTube Educational Channel, the 19th of June 2020. [A first video is about the national meetings for the establishment of the European Network on SCD Patients Organisations](#)

Results:

- 9 patients from Portugal, Italy, Spain, France have given testimony on the video.
- 6 Patients Associations involved: Drépvie, SOS GLOBI Aquitaine, United Onlus, Libera Associazione contro la Talassemia CATANIA, ASAFE - Asociación Española Enfermedad FALCIFORME, APPDH Associação Portuguesa de Pais e Doentes com Hemoglobinopatias
- Here follows some rates obtained from the YouTube EuroBloodNet EDU Channel from the 18th of June 2020 to the 18th of February 2022: Video has been watched 367 times. 1, 3 thousand of times the video thumbnail was shown to viewers since the video was published, and almost 7% views per impressions shown, this measures how often viewers watched a video after seeing an impression.

2nd Testimonies Video. World Sickle Cell Disease International Awareness Day

Another important result to be underlined as patients' testimonies video also for World SCD International Awareness Day is the patients' voice collected in a second Video published on the ERN-EuroBloodNet Youtube Channel: WSCD 2020 EuroBloodNet - SCD Research Prioritization Workshop at ASCAT Video.

The SCD Research Prioritisation Workshop at the Annual Scientific Conference on Sickle Cell and Thalassemia (ASCAT 2019) was a joint project of the ERN-EuroBloodNet together with ASCAT members aiming to

- Engage patients' thoughts in research
- Train patients to Public Patient Involvement in Research
- Train health professionals on a patient research centred approach

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.

The patients presented the workshop outcome, that is to say a top 10 list of topics that what people living with sickle cell disease would like the research community to focus on, to the plenary session of the ASCAT congress, demonstrating that healthcare professionals are willing to change and listen to the patient voice. Testimonies of this experience were gathered in the above mentioned second video.

Results

- 5 patients from: Portugal, USA, Italy, Netherland, Cyprus have given testimony on the video.
- 4 Patients Associations involved: I-X-L, ASAFE - Asociación Española Enfermedad FALCIFORME, APPDH Associação Portuguesa de Pais e Doentes com Hemoglobinopatia, Associazione Pazienti Drepanocitici Padova (AMD)
- Here follows some rates obtained from the YouTube EuroBloodNet EDU Channel: from the 18th of June 2020 to the 18th of February 2022: Video has been watched 273 times. 1,1 thousand of times the video thumbnail was shown to viewers since the video was published, and almost 7% views per impressions shown, this measures how often viewers watched a video after seeing an impression.

SICKLE CELL DISEASE INTERNATIONAL WORLD AWARENESS DAY 2021

ERN-EuroBloodNet has celebrated also the following World Sickle Cell Day. For the 2021 a different approach has been chosen by creating a poster gathering ERN-EuroBloodNet's multi-level actions in the field of Sickle Cell Disease aiming at wide distribution of knowledge and cross border patients support.

Objective

Given visibility to Sickle Cell Disease by creating and disseminating, a poster on all the projects of the ERN-EuroBloodNet dedicated to this rare disease. A cardinal action aiming to raising awareness on Sickle Cell Disease at public at large, scientific community and policy makers.

Methods

The ERN-EuroBloodNet Coordination Team decided the multi-level actions in the field of Sickle Cell Disease to list in the poster.

Design of the poster includes the hypertexts with the direct links to the corresponding SCD ERN-EuroBloodNet actions. This is a way for guaranteeing a comprehensive understanding of each project's objectives and results achieved so far.

Results

The Poster on SCD projects lead by the ERN-EuroBloodNet is downloadable at [ERN-EuroBloodNet website](#)

The poster has been disseminated via: ERN-EuroBloodNet Newsletter and Social Media ([Newsletter](#), [Twitter](#), [LinkedIn](#), [Facebook](#), [Youtube](#)), direct mail to Sickle Cell Experts and SCD Patients Organizations.

Next steps

- Organising the WSCD 2022

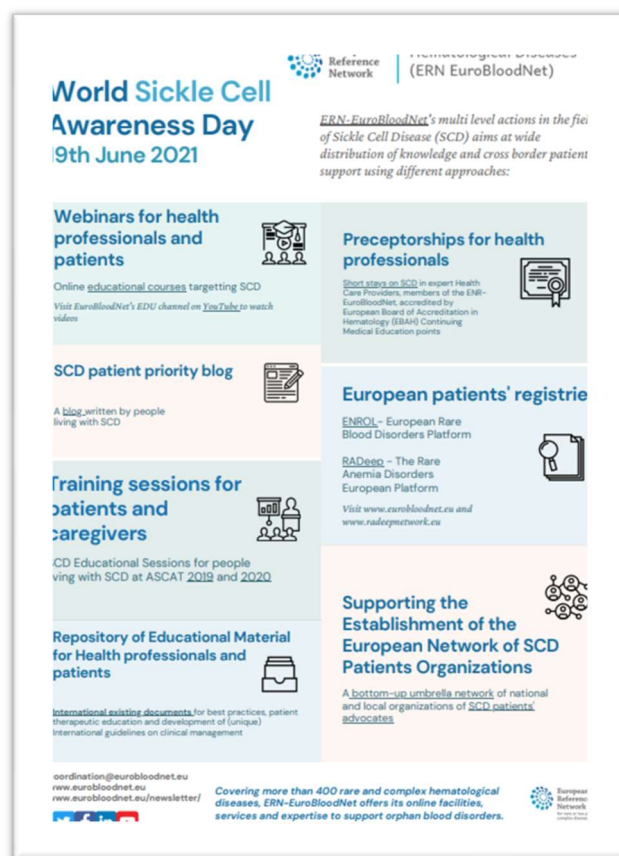


Fig. 7 the poster created for celebrating the WSCD 2021

4.1.3 SICKLE CELL DISEASE PATIENT PRIORITY BLOG

Rationale

In October 2019, with the support of ASCAT, ERN-EuroBloodNet and Oxford Blood Group, 30 people living with sickle cell disease from 13 different countries met together in London at the ASCAT 2019 conference (as described in the Deliverable 6.4 Patients

Action 2020). This forum talked about the needs to be improved for patients with sickle cell disease all over the world. Those needs were prioritized in the Top 10 topics. As for the group, the decision of this blog has set up together.

Objective

- Create a virtual space for people living with Sickle Cell Disease, when they can express themselves about what they consider relevant topics.
- Disseminate the article written in the blog via : ERN-EuroBloodNet Newsletter and Social Media ([Newsletter](#), [Twitter](#), [LinkedIn](#), [Facebook](#), [Youtube](#)), direct mail to Sickle Cell Experts and SCD Patients Organizations, in order to give a good visibility of what patients and caregivers expressed in the blog.

Methods

- ERN-EuroBloodNet Coordination Team together with expert SCD members and supporting partners opened the blog on wordpress and fulfilled with the description of the project
- Invitation to participate to the blog- by writing an article sent to all Sickle Cell Patients, parents patients and patients association in contact with EuroBloodNet. The invitation was sent also to SCD HCPs members, affiliated partners and supporting partners of the ERN-EuroBloodNet in order to disseminate the information among their patients.

Results

The blog is available at [ERN-EuroBloodNet website at Patients advocacy section](#)

- 1st article published on the SCD Patients' Priorities Blog, talks about Chronicles of a Sickle cell & Thalassemic Patient and it is written by a Portuguese patient representative from APPDH (Associação Portuguesa de Pais e Doentes com Hemoglobinopatias).
- 2nd article written by two patients from UK talks about Racism and Sickle Cell Disease
- 3rd and 4th articles both by another Portuguese patient representative from APPDH. The first article analyses the added values of being a patient representative, and the second one shares valuable tips for people affected by Sickle Cell Disease wishing to travel.



Fig. 8 SCD patient Priority Blog section on ERN-EuroBloodNet Website

4.1.4 PILOT OF SICKLE CELL DISEASE PATIENTS CONVERSATION GROUP WITH A PSYCHOTHERAPIST

Rational

Psychological support services are not always available for Rare Disease Patients, for several reasons: Not available in the hospitals, not considered in national health programs, or stigmatization and cultural reticence in being psychologically supported by a health professional.

Sickle Cell Disease is a lifelong condition that put the patients and their families face many psychological challenges. For instance SCD is invisible to other people, but numerous, severe and unpredictable manifestations since very first month of life, the way diagnosis is accepted by the family and reactions to parents perceived by the child could be traumatic, the body image and perception could be complex, among many other reasons.

The project of Conversation Group guided by a psychotherapist for Sickle Disease Patients has been proposed by the Spanish National SCD Patient Association: Asociación Española de Enfermedad Falciforme (ASAFE).

Objective

Promote the organisation of groups discussion at National level to share experiences and problems related to SCD, with the professional support of a psychotherapist (psychologist and psychoanalyst) to help people living with SCD understand and deal with the psychologic consequences of the disease, develop self-confidence and improve their quality of life.

Methods

The project was piloted by ASAFE.

- It has been decided to have two different groups: one for patients and the other for caregivers: parents, brothers and sisters or partner in love, wife and husband.
 - The meetings will last 60-90 minutes
 - Groups will consist of a minimum of 5 and a maximum of 20 people
 - One meeting each 3 months, 4 per year

- Dissemination plan was established for advertising the project: via [ASAFE website](#) and via HCPs. Also flyers will be produced for circulating them among Hospitals.
- A psychotherapist has been selected for guiding the conversation group, the professionals works already with SCD patients in a reference center for SCD, member of the ERN-EuroBloodNet (Hospital General Universitario Gregorio Marañón de Madrid)

Results

- First conversations group started in November
- Next conversation group will be in March

Next step

- A total for 4 conversations for patients and 4 conversation for caregivers will be held during the year



Fig. 9 flyer advertising the SCD Patients Conversation group with Psychotherapist

4.2 TOPIC ON FOCUS FOR PATIENTS AND THEIR FAMILIES OR PATIENTS ORGANIZATIONS

Rationale

ERN-EuroBloodNet is aware of the high impact that the online activities have nowadays, especially in the area of education. Interactive video sessions provided by an expert physicians or a patient advocate in the field, allow patients and caregivers to learn highly specialized knowledge without the need to travel for following onsite trainings. In addition, webinars allow tackling questions gathered from the audience in real time, providing the perfect environment to benefit from the most outstanding experts in the field.

ERN-EuroBloodNet Coordination team together with health professionals leading a specific clinical domain, ePAGs and Eurordis have established a strategy for tailoring the Comprehensive Webinar programs named "Topic on Focus" targeting patients and their families or patients organisations. The strategy is devoted for the definition of comprehensive programs composed of several sessions on very rare diseases given the high necessity for in-depth knowledge sharing.

Objective

It aims to disseminate very innovative topics related to Rare Hematological diseases among patients and their families or patients' organizations in order to give the visibility to the medical services available in Europe, last quality of life cutting-edge advances in the field of this very rare condition and its treatments options.

Methods

Webinars are online educational activities conducted using the Zoom Platform. 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 recordings and the power point presentation will be uploaded and made available on the ERN-EuroBloodNet webpage.

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.

The program's speakers have been chosen among ERN-EuroBloodNet members and supporting partners, expert of the related Rare Hematological Disease.

Topic on Focus' structure

A comprehensive program consisting of a cycle of webinar. Each Webinar lasts 45 minutes: 30 minutes for the expert's presentation and last 15 minutes for hearers' questions.

In the webinars dedicated section on the ERN-EuroBloodNet website, will be soon placed, in educational section: 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 (when those will be available).

The webinar program is advertised in the section dedicated to Patients Webinar > Topic on Focus

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

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!)

- Has the webinar been useful to increase your knowledge on the area?
- Will you disseminate the knowledge among the local patients and parents patients community?
- Would you recommend the webinar among other people living with the same condition?

To promote better recognition of EuroBloodNet's Topic on Focus Webinars, a unique theme was developed including the logo, the look of the powerpoint templates, and videos intro and outro integrating visually this webinars' cycle.

Additionally the webinars are published on the [EuroBloodNet's EDU YouTube channel](#)

Results

Three Topic on Focus Programs: 2 finalized and 1 ongoing. Those programs are described and analysed in the following paragraphs of this Deliverable

4.2.1 TOPIC ON FOCUS ON CUTANEOUS LYMPHOMA FOR PATIENTS' ORGANIZATIONS

ERN-EuroBloodNet Topic on Focus for patients' organizations is coordinated in collaboration with the Lymphoma Coalition, Cutaneous Lymphoma Foundation, France Lymphoma Espoir and Eurordis. It aims to disseminate very innovative topics related to Cutaneous Lymphoma among patients' organizations in order to give the visibility to the medical services available in Europe, last quality of life cutting-edge advances in the field of this very rare condition and its treatments options.



Results

A set of six sessions targeting CL patients' organizations.

Once per month on Monday, from 5 pm to 6 pm (CET), starting from April 2021.

Each session has been moderated by a duo of an expert physician and a patient representative, who has elaborated the session together. The physician has introduced the topic and share the clinical knowledge. The patient representative has ensured that information provided can be well addressed to patients organizations, and highlighted which are the key crucial concepts for patients to the expert and asking to clarify them to the webinar audience. The patient representative moderated the Q&A session.

PROGRAM

- How to connect patients in Europe to CL experts via the ERN (Services available at national or European level, Virtual Board, CPMS) (26 April 2021), Speakers: Prof. Martine Bagot and Pierre Aumont
- Introduction to cutaneous lymphomas, the two prevalent subtypes: Mycosis Fungoides and Sézary Syndrome, Diagnosis and staging (31st May 2021), Speakers: Prof Maarten Vermeer and Mieke de Leeuw
- Introduction to Cutaneous Lymphomas, other subtypes, diagnosis and staging, (28th of June 2021). Speakers: Prof. Antonio Cozzio and Susan Thornton
- Treatment options available in Europe: Skin directed therapy, Systemic therapy and Clinical trials. (19th of July 2021) Speakers : Prof. Rudolf Stadler and Guy Bouguet
- Quality of life: Fatigue, Itch and Skin care, Psychological impact.(13th of September 2021), Speakers: Prof. Julia Scarisbrick and Jenni Burrows
- Impact of treatments on quality of life (18th of October 2021), Speakers Prof. Pietro Quagliano and Keturah Harris.

Program has been recorded and 6 videos implemented in the ERN-EuroBloodNet Edu YouTube channel, and on [ERN-EuroBloodNet website](#)

All the sessions together have gathered more than 245 participants from Europe and outside Europe resulting in average of 41 participants per session.

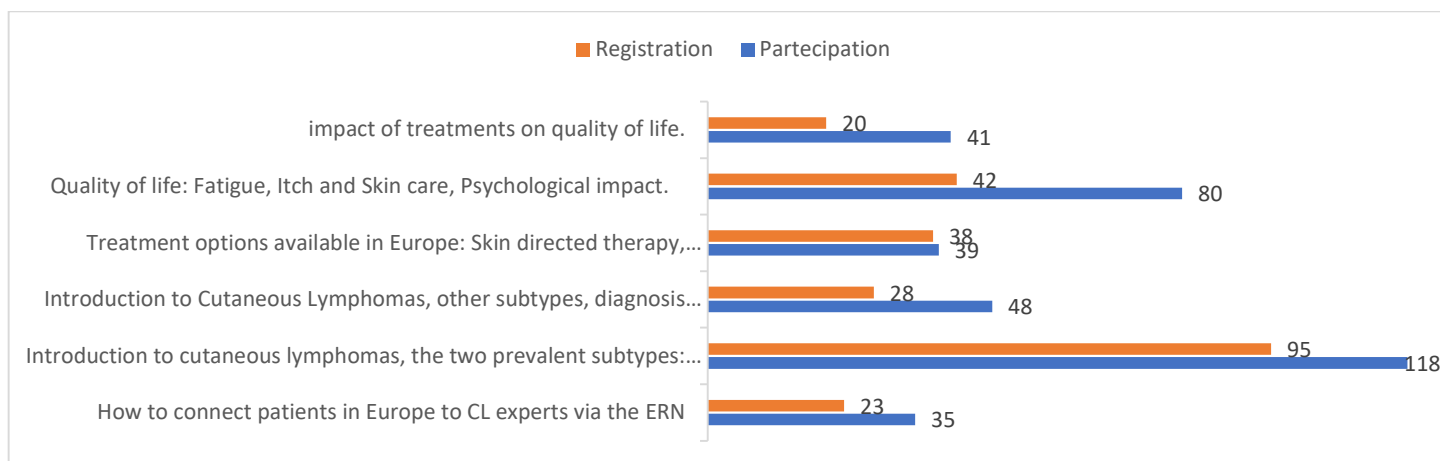


Fig. 10. Number of registered people and number of participant to the Online Session

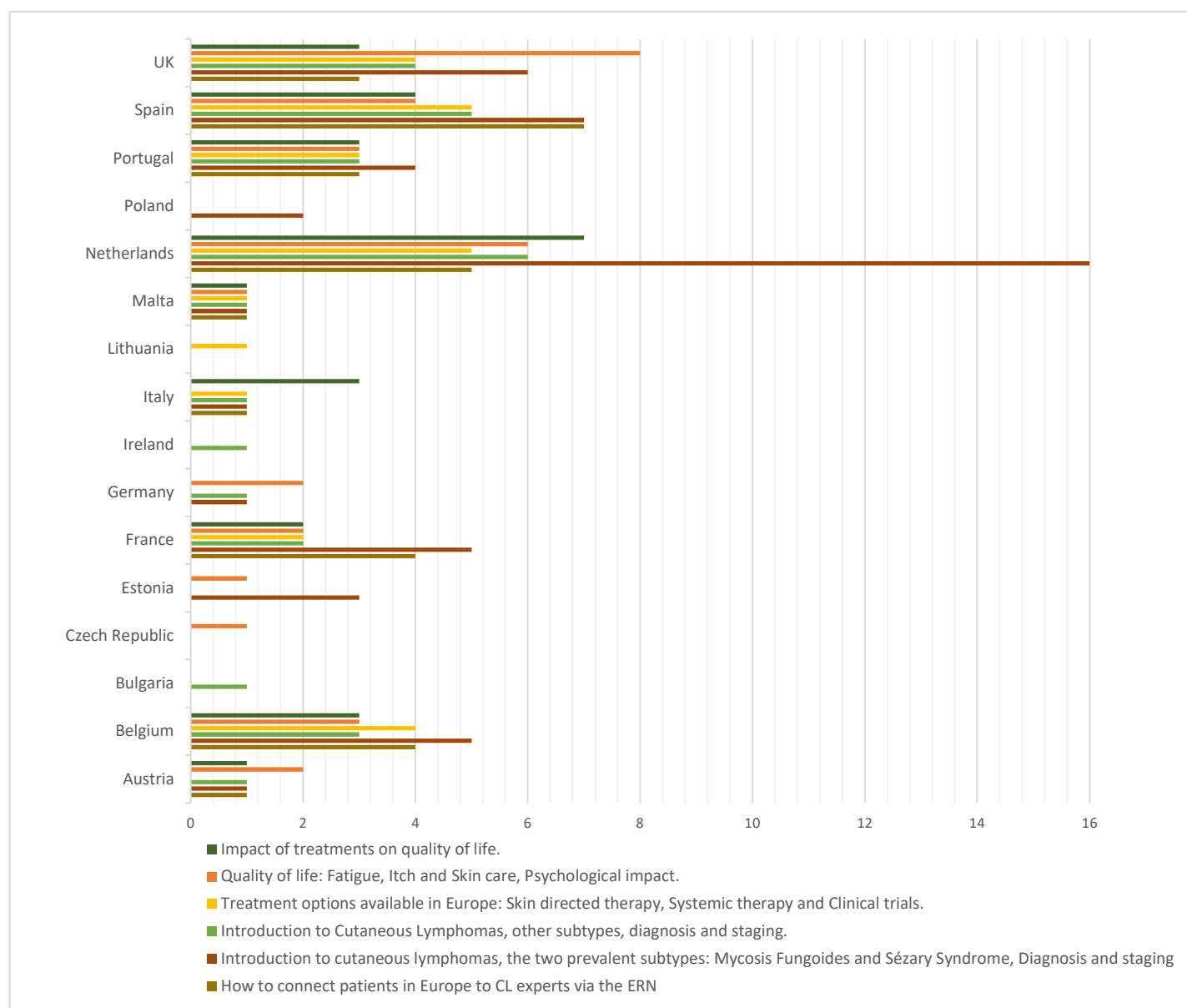


Fig. 11. Comparison of geographical coverage of participants between session - European Countries

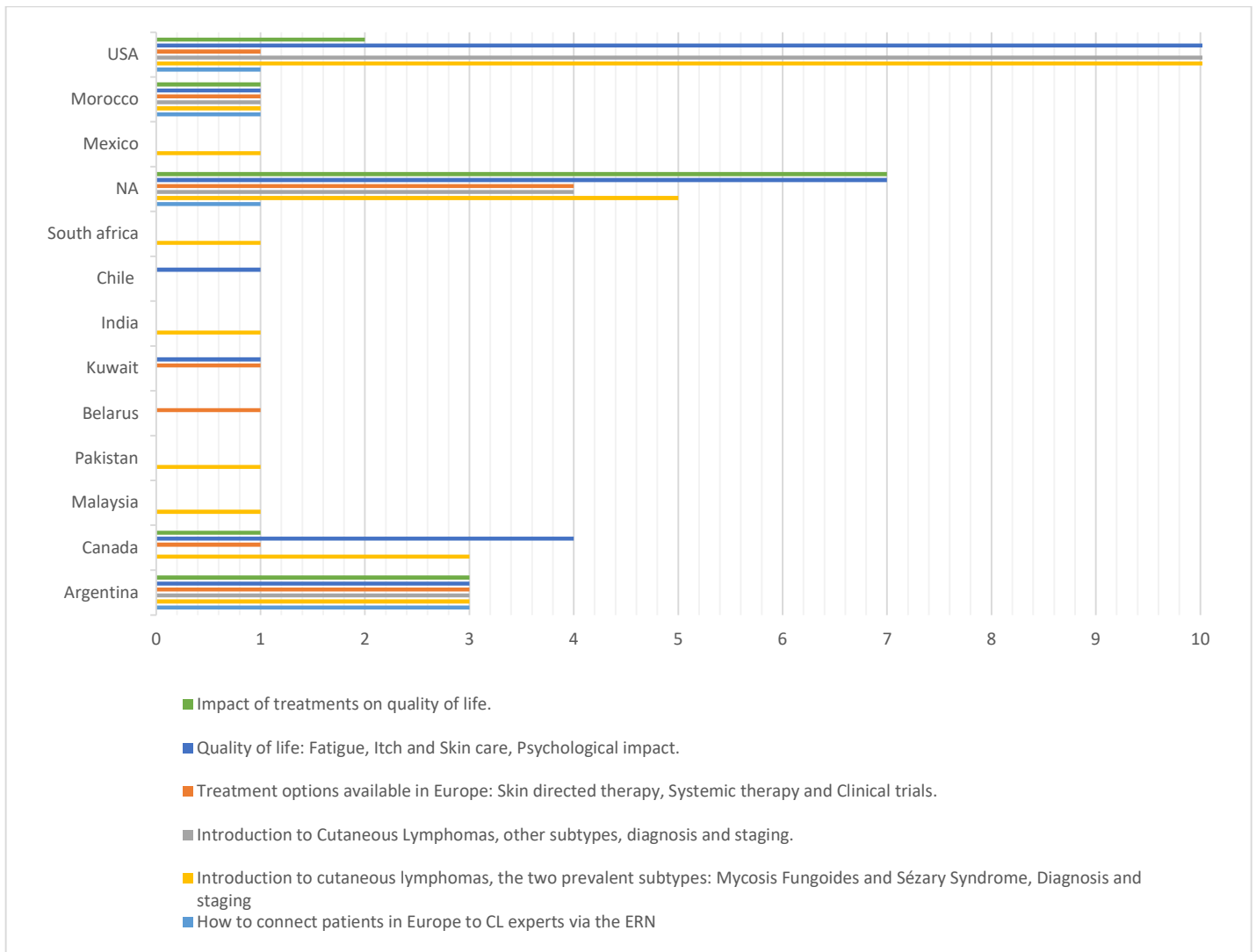


Fig.12 Comparison of geographical coverage of participants between session - Non European Countries

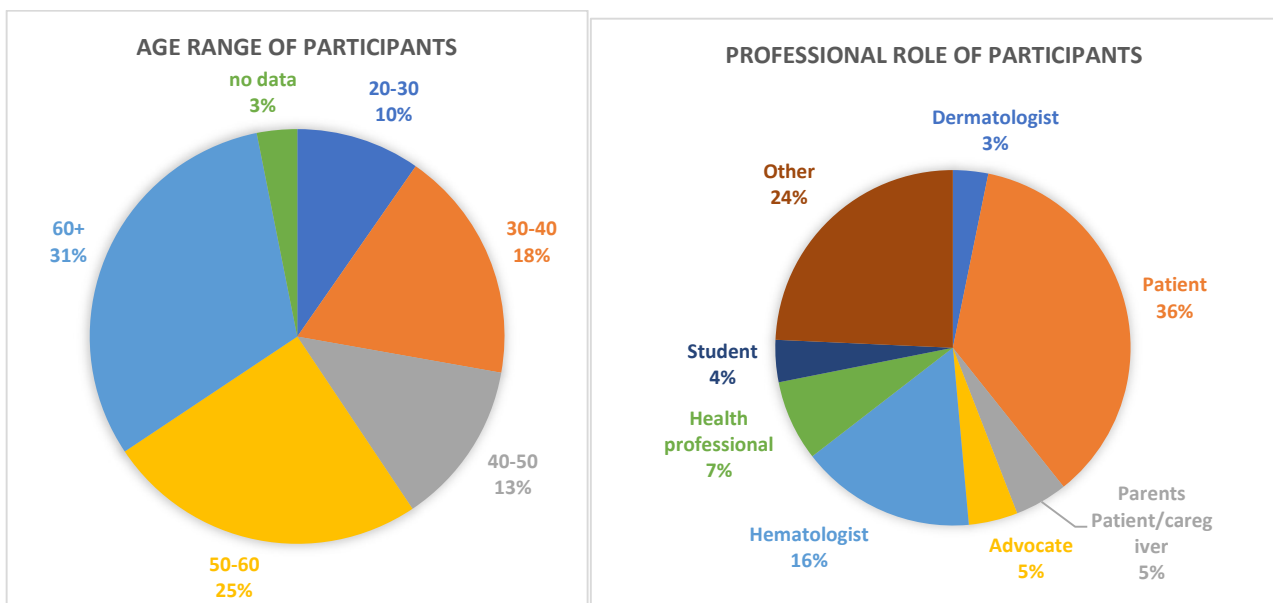


Fig.13. and fig. 14 Age range and professional role of participants

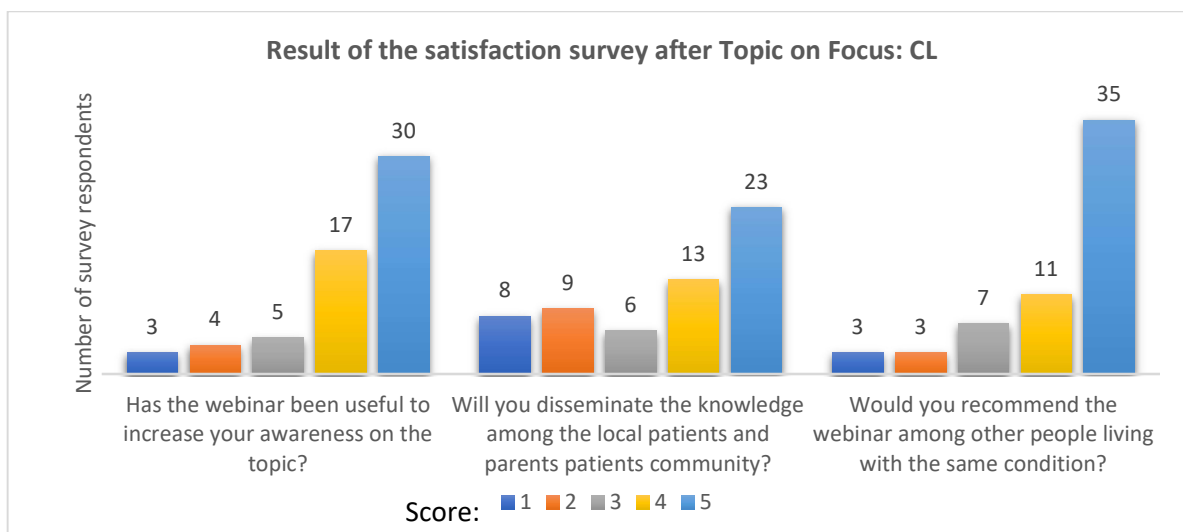


Fig.15 Results of the satisfaction survey submitted to participants after each session of the Topic on Focus on CL. Each question could be rated from 1 (Not at all) to 5 (Absolutely yes!). 59 people answered to the Satisfaction survey. Feedback in general terms was excellent. A total of 88 highest score rates were received.

4.2.2 TOPIC ON FOCUS ON MYELODISPLASTIC SYNDROM FOR PATIENTS AND THEIR FAMILIES

ERN-EuroBloodNet Topic on Focus Myelodysplasies: a topic of interest for patients and their families, is co-organized by the ERN with CCM (the French patient association Connaître et Combattre les Myélodysplasies), the GFM (Groupe Francophone de Myélodysplasies). It aims to disseminate very important topics related to MDS to the patient community and their families: the latest advances in treatment and diagnosis, improving the quality of life in the field of this rare onco disease. This program has been delivered for French Speakers.



Results

A set of 8 webinars targeting MDS patients and their families French speakers.

Once per Month, every Tuesday at 5 pm (CET) starting from June 2021.

Each session has been moderated by a duo of an expert physician and a patient representative, who has elaborated the session together. The physician has introduced the topic and share the clinical knowledge. The patient representative has ensured that information provided can be well addressed to patients organizations, and highlighted which are the key crucial concepts for patients to the expert and asking to clarify them to the webinar audience. The patient representative moderated the Q&A session.

PROGRAM

- Diagnostic et classification des SMD? Date: 1 juin 2021 Orateurs: Pr. Michaele Fontenay, GFM, Annie Kolb, CCM
- SMD de faible risque, Date: 8 juin 2021, Orateurs: Pr. Sophie Park, GFM, Jacqueline Dubow, CCM
- Greffe de Moelle dans les SMD, Date: 6 juillet 2021, Orateurs: Pr. Marie Robin et Pr Didier Blaise, GFM, Raymond Mallarte, CCM,
- SMD de haut risque, Date: 14 septembre 2021, Orateurs: Pr. Pierre Fenaux, GFM, Jacqueline Dubow, CCM, Prédispositions génétique dans le développement d'un SMD, Date: 5 octobre 2021, Orateurs: Dr. Marie Sebert, GFM, Annie Kolb, CCM
- Fatigue et dépression dans les SMD, Date: 23 novembre 2021, Orateurs: Yolande Arnault, IPC, Pascale Chemli, CCM
- SMD et soins de support, Date: 7 décembre 2021 à confirmer, Orateurs: Aurelien Proux, IPC, Pascale Chemli, CCM
- Leucémie myélomonocytaire chronique (LMMC), Date: 11 janvier 2022, Orateurs: Pr. R. Itzykson, GFM, Jacqueline Dubow, CCMM

Program has been recorded and 8 video implemented in the [ERN-EuroBloodNet Edu YouTube channel](#), and on [ERN-EuroBloodNet website](#) including the power points presentations that could be download as PDF.

The program was followed by a total of 248 people, for an average of 35 participants' per session; 408 people registered to the several sessions of the program. As follow:

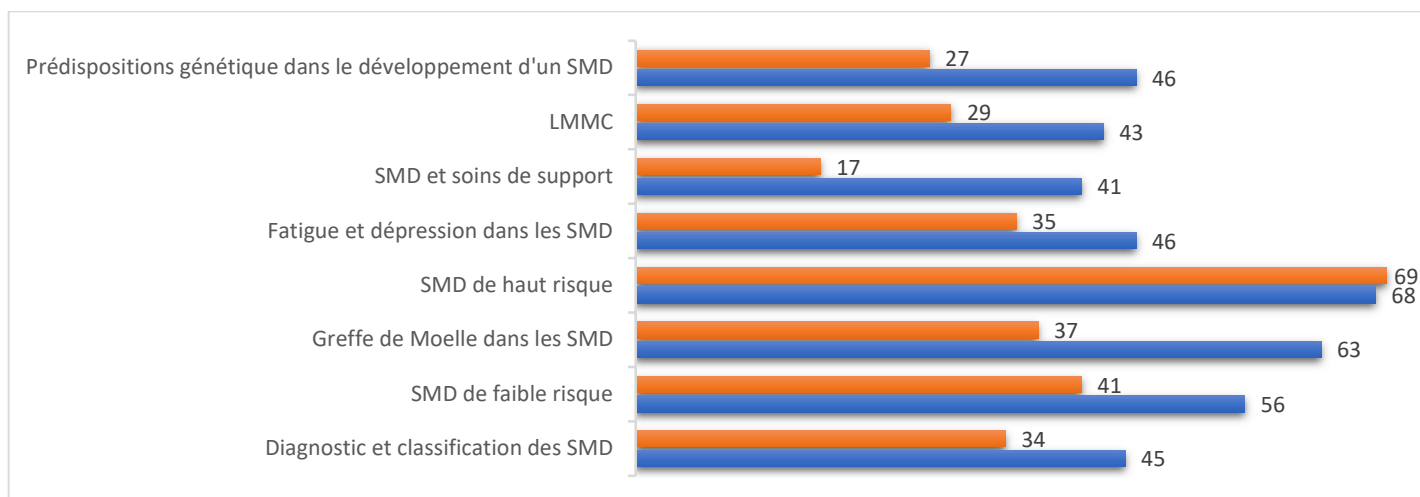


Fig. 16. Number of registered people and number of participants to the Online Session. In blue: number of registered people, in orange number of participants

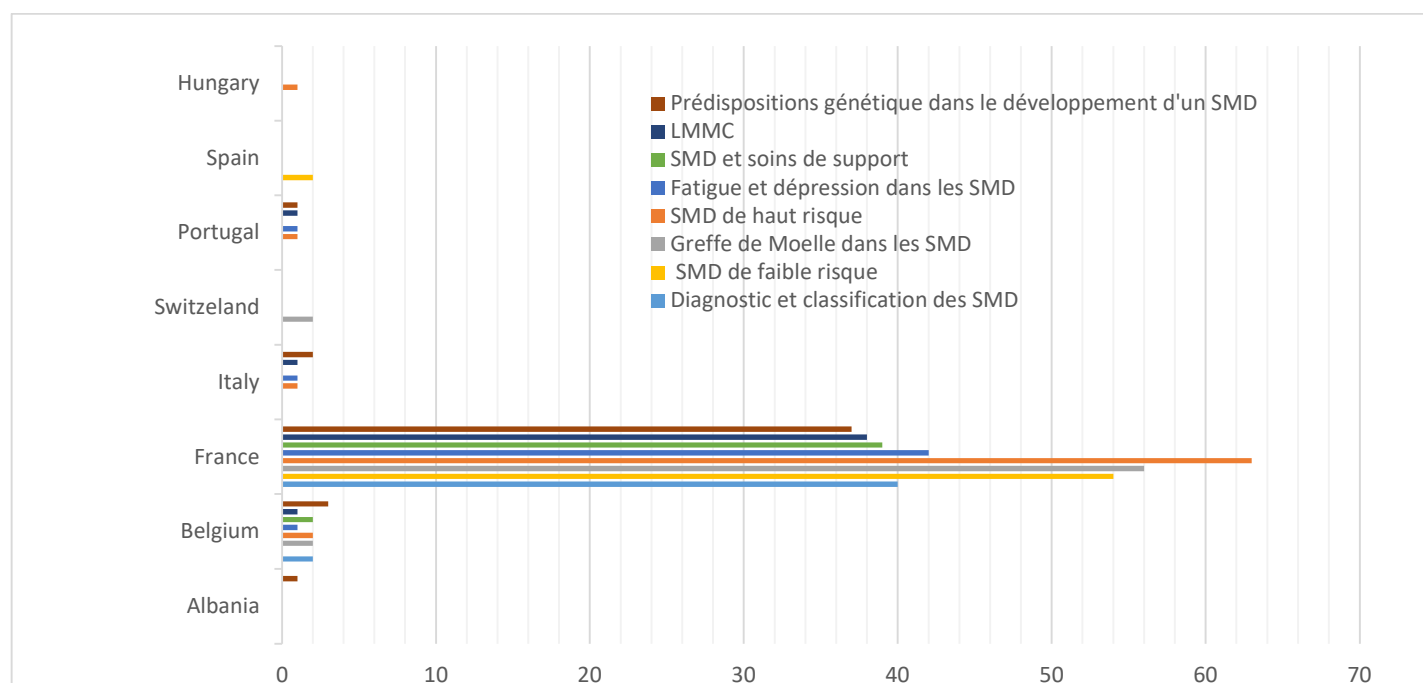


Fig. 17. Comparison of geographical coverage of participants between session - European Countries

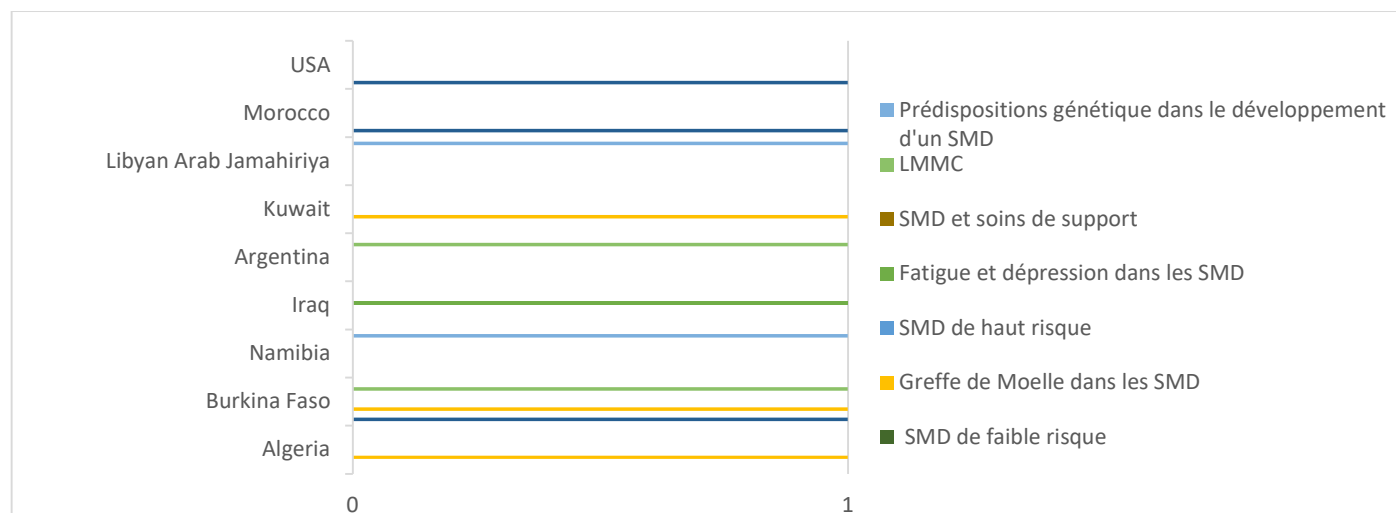


Fig. 18. Comparison of geographical coverage of participants between session - Non European Countries

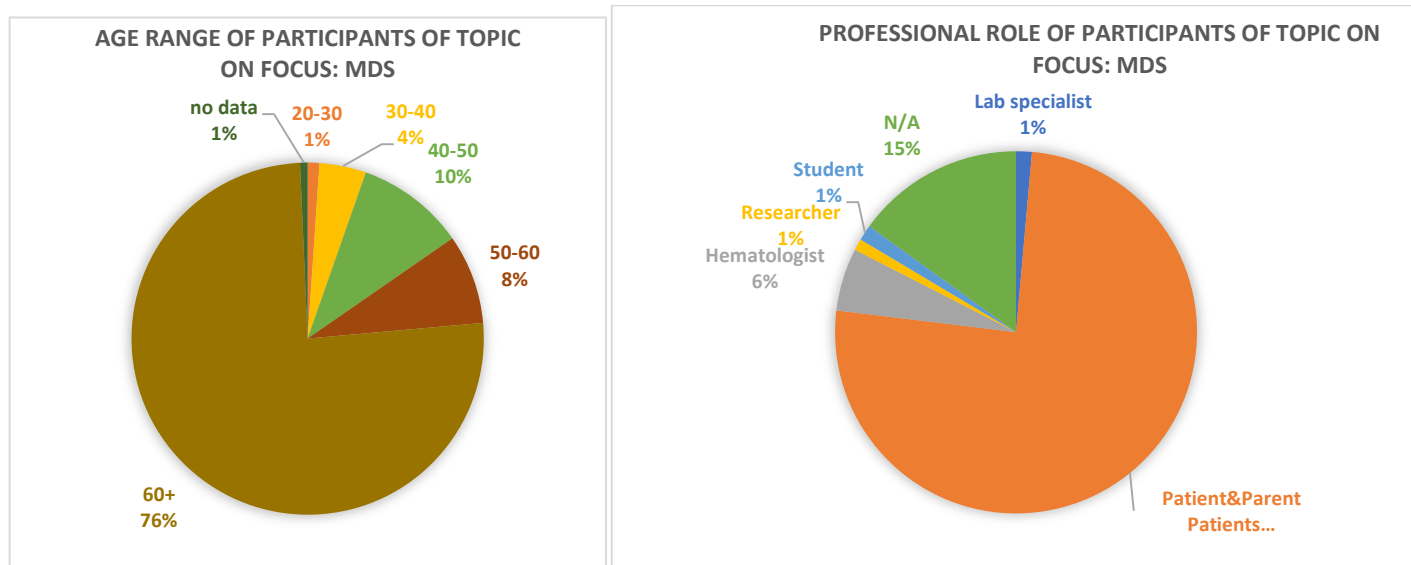


Fig.19 and fig. 20 age range of the registered people and roles

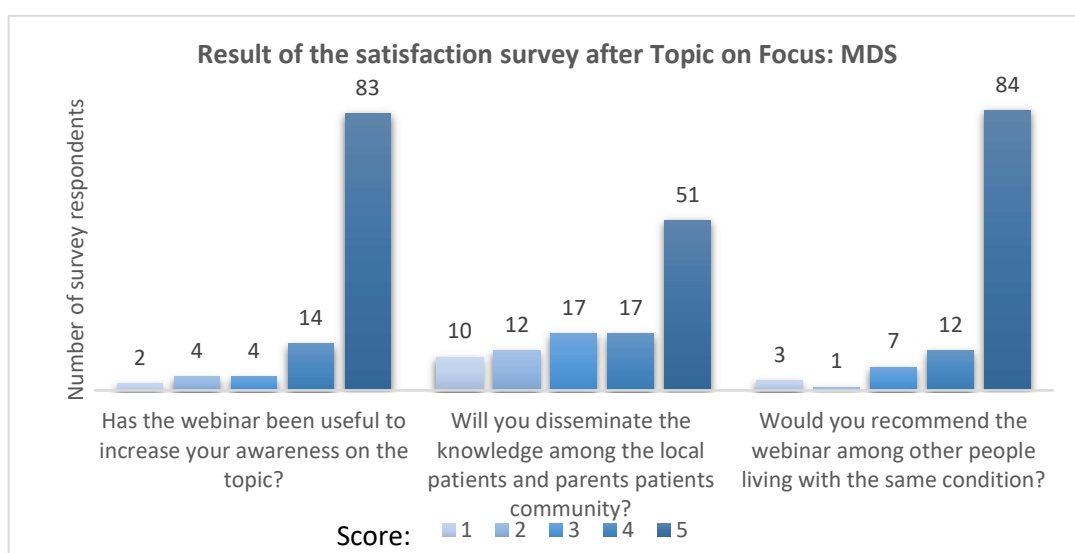


Fig. 21 Results of the satisfaction survey submitted to participants after each session of the Topic on Focus on MDS. Each question could be rated from 1 (Not at all) to 5 (Absolutely yes!). 107 people answered to the Satisfaction survey. Feedback in general terms was excellent. A total of 218 highest score rates were received.

4.2.3 TOPIC ON FOCUS ON SICKLE CELL DISEASE FOR PATIENTS AND THEIR FAMILIES

Results

A set of 11 webinars targeting patients and their families.

Once per month, every Monday 5 pm - 6 pm (Paris CET) starting from January 2022

PROGRAM:

- New therapies for SCD (Date: 31st January 2022), Speaker: Malika Benkerrou (AP-HP Hospital Saint-Louis, Paris)
- Adult Patients Quality of Life (Date: 14th February 2022), Speaker: Noémi Roy, (Oxford University Hospitals NHS Foundation Trust)
- Hydroxyurea and fertility (Date: 14th March 2022), Speaker: Mariane de Montalembert, (AP-HP Hospital Necker, Paris)
- Neurological Damages in SCD, (Date: 4th April 2022), Speaker: Raffaella Colombatti, (AOU Padova)
- Neonatal Screening, (Date: 9th May 2022), Speaker: Béatrice Gulbis, (ULB Hôpital Erasme, Bruxelles)



- Gestational Risk, (Date: 6th June 2022), Speaker: Laure Joseph, (AP-HP Hospital Necker, Paris)
- SCD and immune disease, (Date: 4th July), Speaker: Slimane Allali, (AP-HP Hospital Necker, Paris)
- Genetic Counselling and embryo selection (Date: 26th September 2022), Speaker: Celeste Bento, (Centro Hospitalar e Universitário de Coimbra, EPE)
- BMT: survival infertility and other complications (Date: 17th October), Speaker: Subarna Chakravorty, (King's College Hospital NHS Foundation Trust)
- Polyuria and Enuresis: kidney damages, (Date: 14th November 2022), Speaker: David Rees, (King's College Hospital NHS Foundation Trust)
- Priapism, (Date: 5th December 2022), Speaker: Rachel Kesse Adu, Guy's and St Thomas' NHS Foundation Trust
- First two sessions have been held:
 - New therapies for SCD: 75 participants
 - Adult Patients Quality of Life: 38 participants

Next step

- Following the organization of the programs: 9 upcoming webinars
- Editing and publishing the recorded webinars in order to implement EuroBloodNet' EDU YouTube Channel and the ERN-EuroBloodNet webiste
- Elaborate the analysis of the statistics of this program once it is finalized

4.3. PATIENTS WEBINARS

Rationale

ERN-EuroBloodNet is aware of the high impact that the online activities have nowadays, especially in the area of education. EuroBloodNet's Patients Webinars are led by experts in the field of Rare Hematological Diseases, health professionals and patients representatives. Each webinar session provides patients community, patients' organisations, caregivers and health professionals with highly specialized knowledge on a specific Rare Hematological disease or groups of disease. In addition, it allows to tackle questions gathered from the audience in real time, providing the perfect environment to benefit from the most outstanding experts in the field.

Objective

EuroBloodNet's Patients Webinars are led by experts in the field of Rare Hematological Diseases, health professionals and/or patients representatives. This program consist of one webinar that provides patients community, patients' organisations, caregivers and health professionals with highly specialized knowledge on a specific Rare Hematological disease or groups of disease. In addition, it allows to tackle questions gathered from the audience in real time, providing the perfect environment to benefit from the most outstanding experts in the field.

Method

Patients' representatives or health professionals that collaborates with the Network propose topics to the ERN-EuroBloodNet. So, webinar should tackle an identified educational need. In addition, a survey could be conducted among those associations that collaborate with the ERN in order to identify possible topic to be addressed by a webinar. Webinar target is: patients community, patients' organizations, caregivers and non expert health professionals. Speakers are identified among ERN-EuroBloodNet members (ePAGS, physicians and active patients associations)

Webinar structure

Each Webinar lasts 45 minutes: 30 minutes for the expert's presentation and last 15 minutes for hearers' questions.

In the webinars dedicated section on the ERN-EuroBloodNet website, will be soon placed, in educational section: a brief introduction and aims of the patients' 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 (when those will be available).

The webinar program is advertised in the section dedicated to Patients Webinars. Registration form includes the following fields mandatory to receive the link for connection:

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!)

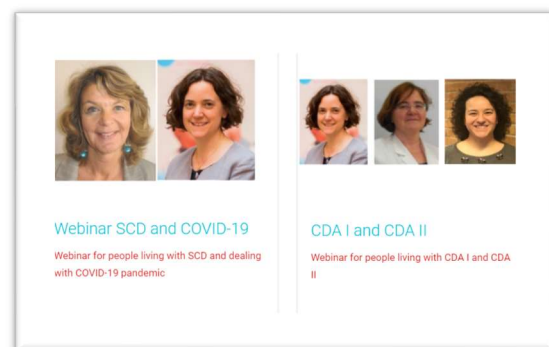
- Has the webinar been useful to increase your knowledge on the area?
- Will you disseminate the knowledge among the local patients and parents patients community?
- Would you recommend the webinar among other people living with the same condition?

To promote better recognition of EuroBloodNet’s Patients Webinars, a unique theme was developed including the logo, the look of the powerpoint templates, and videos intro and integrating visually this webinars’ cycle.

Additionally the webinars are published on the [EuroBloodNet’s EDU YouTube channel](#).

Results

Two Patients Webinar, described in the next two paragraphs of this Deliverable



4.3.1 PATIENT WEBINAR: COVID-19 AND SICKLE CELL DISEASE

The ERN-EuroBloodNet faced unprecedented challenges to gather and spread information on the correct treatment and management of infection by Coronavirus (COVID-19) in patients affected by Rare Hematological Diseases. Information gathered are accessible on the [Network’s Website](#). The ERN is also helping patients affected by Red Blood Cell Disorders who are also infected with the new coronavirus to receive care and cure adequate to their specific situation by establishing a [ERN-EuroBloodNet Collaborative Platform on Red Blood Cell and COVID-19 patients](#). Finally, the ERN has dedicated a specific educational session on COVID-19 addressed to Sickle Cell Disease patients and parents' patients during ASCAT 2020.

During this experience, that has gathered more than 40 SCD patients representative worldwide, the ERN-EuroBloodNet has reached the outcomes that patients find difficult to know who/what information to trust about COVID-19 infection. They felt fear and anxiety about how to handle the daily life and respect the barriers gestures, and even they did not know if getting hospitalized during vaso-occlusive crises would have been good or bad for them in the context of the coronavirus pandemic. Finally, they wished to know the data on people with SCD getting affected.

This is the reasons why the ERN-EuroBloodNet has organized a webinar dedicated to SCD and COVID-19 infections addressed to SCD Patients and parents’ patients.

Webinar was led by two clinicians at the front line dealing with the COVID-19 pandemic and SCD adult and paediatric patients, Prof Mariane de Montalemebert (SCD paediatric specialist) and Dr Noémi Roy (SCD adult specialist). The aim is to support people living with SCD, families and caregivers that are currently dealing with COVID-19 case.

Questions patients community would like to addressed to physicians related to COVID-19 have been previously collected during the SCD Patient Educational Session at ASCAT 2020 (event described in a dedicated paragraph of this deliverable). Additional questions have been gathered via a survey circulated among SCD patients community before the webinar.

Content of the Webinar

- Symptomatic and asymptomatic cases of COVID19 in children and adults affected by SCD
- Risks for children and adults affected by SCD
- How to live and deal with barrier gestures for children and adult affected by SCD
- Vaccination on COVID19 for children and adults affected by SCD

Results

40 participants followed the webinars from 64 registered to the event

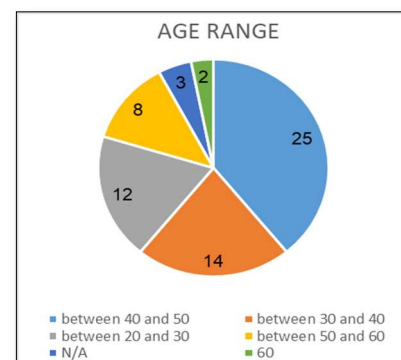
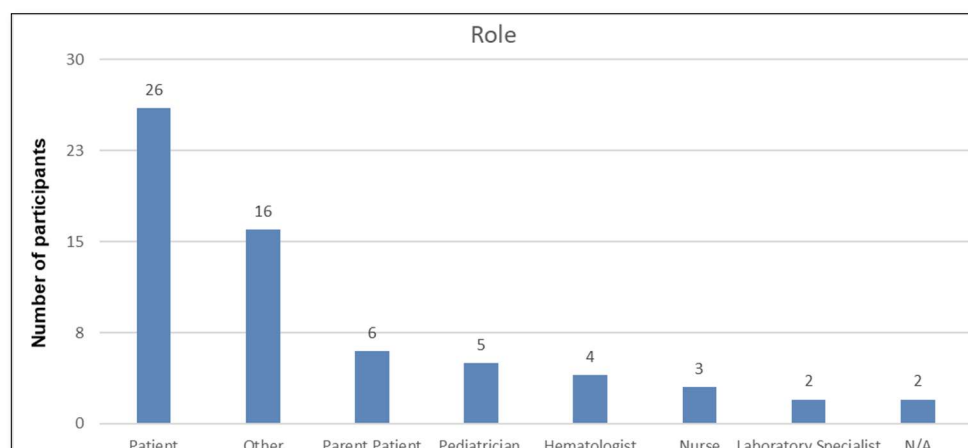


Fig 22. Role of the attendance. The majority of participants were SCD patients (26), followed by other roles (6 participants, as Rare Disease Network project manager), 5 people parents patients, 9 physicians (5 pediatrician and 4 hematologist), 3 nurses, 2 Laboratory Specialist and 2 N/A.

Fig 23. Details of the number of attendants per age range. Attending to age range, the higher representation at the webinars are participants between 40 and 50 age range.

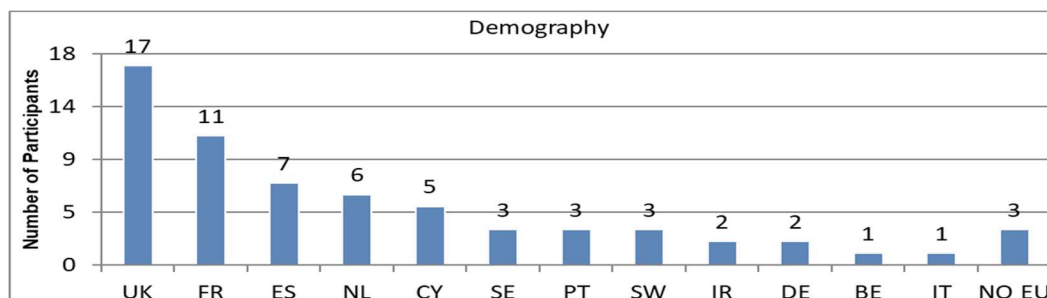


Fig 24. Country coverage of the attendants to the webinar on Covid19 for people living with SCD. Most of participants came from UK, following France, Spain and Netherland. 3 participants were from USA, Israel and Trinidad and Tobago

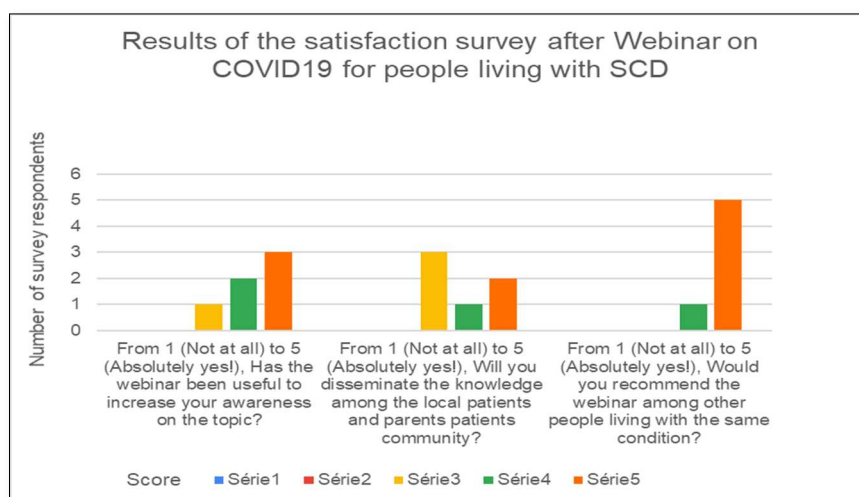


Fig 25. I Results of the satisfaction survey submitted to participants after the webinar. Each question could be rated from 1 (Not at all) to 5 (Absolutely yes!). In the table you can see that 6 participants answered to the satisfactions survey. Feedback in general terms was excellent. A total of three "9" rates were received.

Video of the Patients Webinar: SCD and COVID-19 infections addressed to people living with SCD . The recorded webinar is publicly accessible on the [ERN-EuroBloodNet YouTube Channel](#)

4.3.2 PATIENT WEBINAR: CONGENITAL DYSERYTHROPOIETIC ANEMIA I AND CONGENITAL DYSERYTHROPOIETIC ANEMIA II

Rationale

The ERN-EuroBloodNet Webinar addressed to people living with Congenital Dyserythropoietic Anemia I (CDA I) and Congenital Dyserythropoietic Anemia (CDA II) is in collaboration with Association Anémie Dysérythropoïétique Congénitale (A.A.D.C), the French Patients Association representing Congenital Dyserythropoietic Anemia and EURORDIS. Request for organizing a webinar addressed to people living with CDA I and CDA II provides patients' community, patients' organisations, caregivers and health professionals with specialized knowledge on CDA I and CDA II, came from the A.A.D.C patients association.

Content of the webinar

- General introduction on CDAs (including epidemiology), Dr Noemi Roy
- Diagnostic aspects, Dr Paola Bianchi
- Clinical aspects, Dr Roberta Russo
- Current and future therapeutic perspectives, Dr Noemi Roy

Results

42 participants followed the webinars, 42 registered to the event

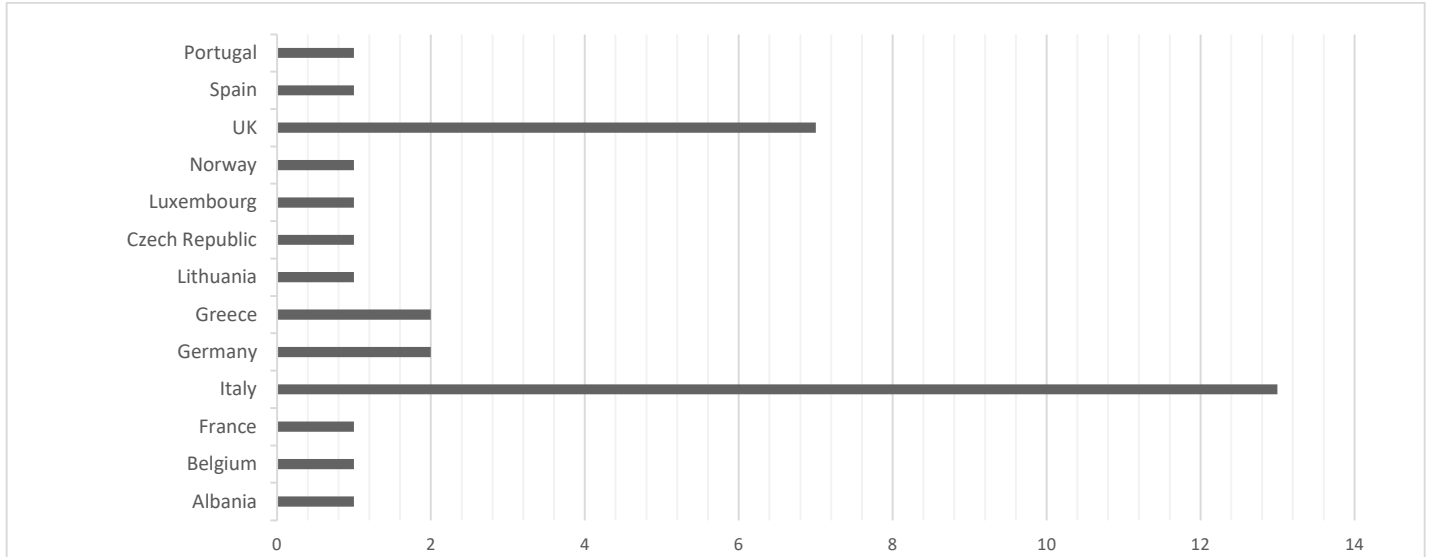


Fig. 26 the Geographical Coverage of participants to the Webinar - European Countries

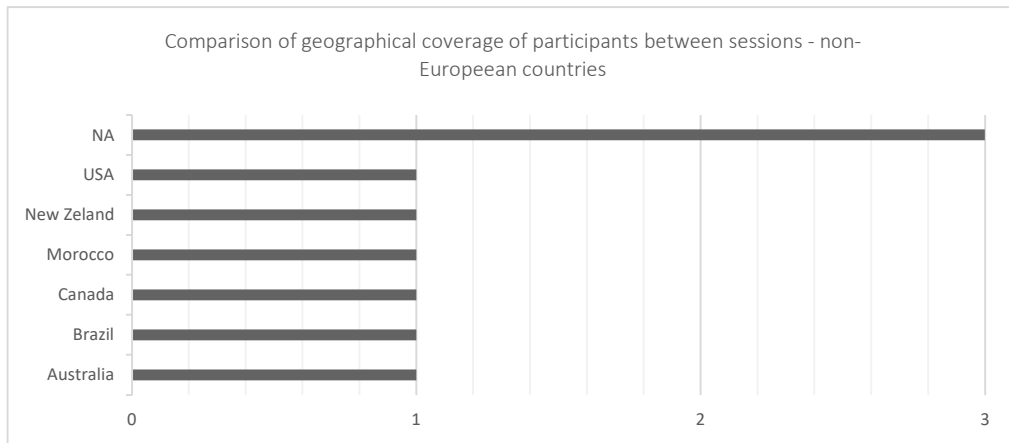


Fig. 27 Geographical coverage of participants: non-European Countries

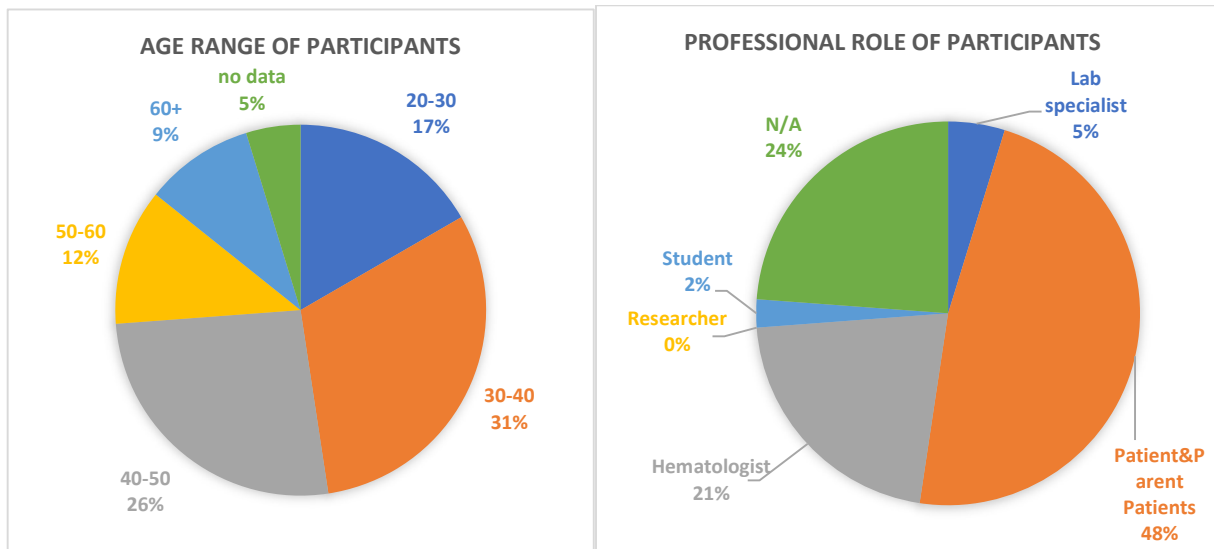


Fig. 28 and fig. 29: Age range and role of participants to the webinar



Fig 30. I Results of the satisfaction survey submitted to participants after the webinar. Each question could be rated from 1 (Not at all) to 5 (Absolutely yes!) 16 participants answered to the satisfactions survey. Feedback in general terms was excellent.

Video of the Patients Webinar: CDA I and CDA II. The recorded webinar is publicly accessible on the [ERN-EuroBloodNet YouTube Channel](#).

5 . OVERVIEW ON FORTHCOMING PATIENTS' PROJECTS

The following projects have been proposed via a joint strategy among the ERN-EuroBloodNet Coordination Team, ePAGs and EURORDIS for being carried on during next annuals plans.

1. Establishing a Comprehensive Webinar program for patients on Bone Marrow Failures Syndromes and on Castleman Disease
2. Organising others patients webinars (webinars consisting of only one session) and identifying new niches of intervention
3. Producing Educational Infographics based on a) research results / b) treatments options & Guidelines --- return to patient
4. Creating Patients summary based on Clinical Practice Guidelines. It has been suggested to create it in line with the task of establishing Guidelines on Burkitt Lymphoma.
5. Establishing Patients Pathways. It has been proposed the possibility to establish a patient journey on Cutaneous Lymphoma. Another non oncological clinical area will be identified
6. Coordination of patients educational and advocacy trainings
7. Coordination of patients' sessions at congresses, as for instance the organization of ASCAT 2022 in October
8. Participating to international Rare Disease Campains
9. Creating a section on the ERN-EuroBloodNet website for assisting Rare Hematological Disease Patients dealing with Cross Border Health procedures or searching for an expertise abroad

6 . CONCLUSIONS

Thanks to the close collaboration with EURORDIS, European Patients Networks and Patients organizations at National and local level, the ERN-EuroBloodNet has reached relevant outcomes in promoting patients-centered projects. The axes explored by ERN actions in the field of Rare Hematological Diseases during the fourth period of the Network for patients empowerment targeting patients and their families or patients associations are:

- Patients advocacy's actions
 - The identification of new ePAGs advocates, in order to promote patients' rights, access to information and healthcare services at European Level.
 - The creation of sections on the ERN-EuroBloodNet website dedicated to patients organizations supporting the ERN and the related projects they are contributing
 - Facilitating the creation of a bottom up European Network of SCD Patients Organizations, and the kick off meeting organized for 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.
- Therapeutic Patients Educations
 - Online webinars programs that reached a worldwide audience and rated as excellent by the participants.
 - Single webinar covering need expressed by patients themselves that achieved great outcomes: international audience and scored as excellent.
 - Putting the bases for the creation of videos e-learning repository under the frame of Connecting EuroBloodNet (CEF Telecom Grant)
- SCD related actions :
 - Sickle Cell Disease Patients Educational Session at ASCAT 2020 and ASCAT 2020 (January): an important opportunity for SCD patients and parents patients to learn more about the latest advances in terms of diagnosis, treatment and emerging new therapies in the field of the haemoglobinopathies, creating a global patients group that can share opinion on burdens of living with SCD, sharing best practices and advocating at European and Global level.
 - ERN-EuroBloodNet participating to the World Sickle Cell Day 2020 and 2021, for making SCD patients' needs visible at public at large, policymakers and scientific community
 - Sickle Cell Disease Patient Priority Blog, a valid instrument that gives to patients a space for expressing themselves, while training them to communicational skills
 - Pilot of Sickle Cell Disease Patients Conversation Group with a psychotherapist, an ambitious project that contribute in raising awareness on mental health and fight the cultural reticence against mental healthcare

In conclusion, ERN-EuroBloodNet Patients-centred projects achieved good outcomes in empowering people living with RHDs. Several diseases' areas have been tackled by addressing educational programs, by organising advocacy trainings. The collaboration with patients organizations and patients representatives has reached a good visibility on the ERN-EuroBloodNet website. Several ambitious projects have been launched: facilitating the creation of an European Network of Sickle Cell Disease Patients organization or dealing with Cross Border Health assistance. In addition, the ERN-EuroBloodNet has increased its area of Patient Therapeutic Education interventions by creating a new project on mental health too. Finally, new projects has been proposed for implementing the ERN's patient-centred strategy: the production of patients' summary for guidelines, the creation of patients' journeys and their related infographics supporting documents.

ANNEX I

ASCAT 2020

DELIVERABLE 6.5 ERN-EUROBLOODNET REPORT ON ACTIONS FOR THE PROMOTION OF PATIENTS' EMPOWERMENT 2



**European
Reference
Network**

for rare or low prevalence
complex diseases

 **Network**
Hematological
Diseases (ERN EuroBloodNet)

AGENDA

SCD PATIENTS' EDUCATIONAL SESSION ASCAT 2020 (26th – 31st October)

26th of October 2020

5 pm- 7.40 pm (PARIS CET Time)

1. (5pm - 5.10 pm) Welcome&Opening

Moderated by Raffaella Colombatti, Mariangela Pellegrini and Agnes Nsofwa

- a. Recap on last year's meeting including ASCAT video produced by EuroBloodNet containing participants' testimonies and workshop results.

2. (5.10 pm – 6.30 pm) Webinar

Moderated by Noemi Roy and Funmi Dasaolu

- a. **(5.10 pm – 5.20 pm) Pre-recorded session** with the COVID patients experience from London

→ 2 patients testimonies from London :

Reia Costa (UK)

Ayodele Ibidapo-Obe (UK)

- b. **(5.20 pm – 5.30 pm) Pre-recorded session** with EuroBloodNet representatives about how patients with SCD coped during the lockdown, access to treatment, services, follow up

→ 3 patients testimonies from Europe

Andreas Athanasiou (CY)

Diabana BA (FR)

Decio Temporario (PT)

- c. **(5.30 pm - 5.40 pm) Live Session Voluntary/Third sector**

Discussion on the role of Voluntary/Third sector during the pandemic (10 minutes).

→ 1 SCD patients representative testimonies

N' Zallah B. Zamani (Nigeria)

(5.40 pm- 5.50 pm) 10 min break

d. **(5.50 pm – 6.30 pm) Debate** (Summary of key messages + questions raised during testimonies + moderators gather questions written in the chat)

3. **(6.30 pm – 6.45 pm) Peer reviewing experience in UK**

Moderated by Noemi Roy and Sharon Brown-Peters

a. **Pre-recorded session** by a patient who would like to share their experience (5 min).

→ 2 SCD patients representative testimonies

Vanessa Wills (UK)

June Okochi (UK)

b. **Q&A session.** (Summary of key messages +pre-identify questions raised by testimonies +some questions in the chat) (10 min).

(6.45 pm – 6.55 pm) 10 min break

4. **(6.55 pm - 7.25 pm) Why I took part in research)**

Moderated by Noemi Roy and Miriam Santos

a. **Pre-recorded session** by a patient who would like to share their experience

→ 3 SCD patients testimonies

Kuseke Sona Luce (FR)

Miriam Santos (Portugal)

Oluyemi Moses (USA)

b. **(7.25 pm – 7.40 pm)Q&A Session** (Summary+ Q&A pre-identify questions raised by testimonies +some questions in the chat). (15 min)



28th of October 2020

5 pm – 7.40 pm (PARIS CET Time)

5. (5pm – 5.50 pm) “Meet the expert” session with live Q&A
Moderated by: Raffaella Colombatti, Subarna Chakravorty, Esther Owuta Pepple Onolememen

Panel of physicians: 2 paediatricians, 2 adults haematologist

- a. **Topics identified with a survey submitted to SCD patients**
- Newborn and infant SCD Screening, **Stephan Lobitz, Germany**
 - Neurological Complications in SCD, **Maddalena Casale, Italy**
 - Adult Patients Quality of Life, **Jeremy Anderson, UK**
 - BMT: survival infertility and other complications, **Jean-Hugues Dalle, France**
 - New therapies for SCD, **John Brewin, UK**

(5.50 m – 6 pm) 10 min break

6. (6 pm – 6.50 pm) “Meet the patient” 2 parallel sessions with live Q&A

1st Parallel session “Meet the patient”: Physicians addressing questions to adult patients
Moderated by: Subarna Chakravorty and Biba Tinga

Panel of Patients:

Ibidapo-Obe (UK)

Kuseke Sona Luce (FR)

Cassandra Timnel (USA)

2nd Parallel session “Meet the patient”: Teenagers patients addressing questions to adult patients

Moderated by: Raffaella Colombatti and Wilhermina Naa Koshie Lamptey

Panel of Patients:

Awa Kekeh (FR)

Marsha Mothersill (UK)

Michele Nkoudou (IT)

(6.50 pm - 7 pm) 10 min break

7. (7 pm – 7.40 pm) ‘My life, my word’

Moderated by: Mariane de Montalembert and Sola Njoku

- a. **Pre-recorded session:** paediatric or young patients express their view/questions about their life with SCD.
Devard Jones (UK)
Kaltouma MAHAMAT NOUR (FR)
Judy Grandison (Canada)
Gutam Dongre (India)

- b. **Q&A session** (pre-identify questions raised by testimonies +some questions in the chat 15 min)

31st of October 2020

Moderated by: Beverley Francis-Gibson and Anie Kofi

8. **(2 pm- 3.15 PARIS CET time pm) Q&A session** (Question raised in previously sessions but not enough addressed + new questions and comments) (1h)

9. **Plenary session (18.30 PARIS CET Time – 21.10 PARIS CET Time)** (Presentation of key messages and results of the SCD Patients Educational Session at ASCAT 2020 Plenary Session as conclusion of the Congress.) (10 min)

**“We can’t do this without you,
you can’t do this without us”**
(From G. Eda Ömür testimony)

Participants of the ASCAT SCD 2020 people affected Session





1

Last Year

- A wonderful group of clinicians from all around Europe who support the **meaningful involvement** of people affected worldwide
- Capacity to improve information, build collaborations, influence research and impact on care models
- Presentation of the results of the SCD Research Prioritization Workshop at the audience of ASCAT 2019




SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

2

This Year

2020.....Not in person.....





.....BUT.....
**STILL CONNECTED
AND
NOT ALONE**




SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online


3

**49 people affected and
Parents:**





- 1 CYPRUS
- 4 SPAIN
- 7 FRANCE
- 2 IRELAND
- 5 ITALY
- 2 NETHERLANDS
- 3 PORTUGAL
- 1 SWITZELAND
- 13 UK
- 1 AUSTRALIA
- 2 CANADA
- 1 INDIA
- 2 NIGERIA
- 5 USA

38 from European countries



13 experts: physicians, nurses, psychologist; Eurordis and ERN representatives

SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

4



5

Key features of the patient workshop

- Legacy from 2019 ASCAT - we are building on strong foundations
- Moderators included healthcare professionals *and people affected*
- **Topics chosen:**
 - Had been identified by people affected as being important for research at the 2019 ASCAT
 - Had been prioritized by people affected as requiring more information from a EuroBloodNet Survey
- “Meet the expert” was not only meeting medical professionals but also people affected as experts of their condition
- All people affected who participated had opportunities to ask questions in chat and/or live
- Mixture of live and pre-recorded presentations

European Reference Network ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

6

Day 1-26th October

- COVID session
 - Testimonies from different countries
 - Live Q&A
- PEER REVIEW session
 - Testimonies from UK patient participants
 - Live Q&A
- WHY I TOOK PART IN RESEARCH session
 - Testimonies from different countries
 - Live Q&A

European Reference Network ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

7

COVID session outcomes




- Experience varied between countries (shielding vs not)
- Quality of care varied between countries
- Some experienced the “best treatment ever” in hospitals!
 - Direct phone access to clinicians
 - Rapid clinical assessment
 - Admission to correct (haematology) ward
- Some employers were surprised to see SCD on the list of high risk
 - it was validating for a lot of people to have the seriousness of condition finally recognized by government, employers, colleagues, friends
- Fear and anxiety was commonplace
- Use of telehealth- quite a good legacy for the future
- Role of social media in staying informed
 - Access to information – but what/who do you trust?

European Reference Network ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

8

PEER REVIEW session outcomes

- What is peer review?
 - UK system for quality assessment of services- healthcare providers assess each other's services
 - Patient representatives are included- they review the documents, speak to healthcare providers and to users of that service to get a subjective view
- Testimony from 2 people who had been patient reps on peer review process
- Lots of curiosity about the process from those who were not familiar with it
 - Other countries
 - People in the UK as well did not know
- We liked that we can hold stakeholders accountable
- How can we spread this practice of Peer Review?
 - could this be done elsewhere as well?
 - Could EuroBloodNet be able to spread this practice
- Key messages from peer review:
 - How do we advertise this?
 - How do we share good practice?
 - Why do people still not have good care when we have this process (sometimes due to resources)







SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

9

WHY I TOOK PART IN RESEARCH session outcomes

- 4 testimonies from people who had participated in different types of research (BMT, new drug, 'old drug', telephone questionnaire)
- Strong message:
 - The heavy emotional and psychological toll on research participants
 - Even the clinicians in the room were surprised- it made them think *how to do this better in future and support people affected better when they are research participants*
- Lack of advertising:
 - how do you find out and get involved?
 - How do you spread awareness about research
 - What about countries where there is research but poor access
 - What about countries where there is no or little research happening?
- People struggle with the idea of placebo
- Two main problems:
 - Far fewer trials in children
 - Mental health- we need more research!








SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

10

Day 2 - 28th October

- Meet the **medical expert** sessions
 - Newborn and infant SCD Screening, **Stephan Lobitz, Germany**
 - Neurological Complications in SCD, **Maddalena Casale, Italy**
 - Adult people affected Quality of Life, **Jeremy Anderson, UK**
 - BMT: survival infertility and other complications, **Jean-Hugues Dalle, France**
 - New therapies for SCD, **John Brewin, UK**
- Live Q&A
- Parallel Meet the expert session: moderators 1 physician/1patient
 - **Young people affected** addressing questions to adult people affected
 - **Physicians** addressing questions to adult people affected
- SCD: "My life, My words"
 - Testimonies from pediatric patient and parents from different countries (France, India, Canada, UK).

SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

11

Meet the medical expert session outcomes

- Not enough time!
 - People had many questions
 - Lots of unanswered questions- still in the chat.
 - People would like more opportunities to ask questions to the experts




- EuroBloodNet will start some seminars
 - We know there is a concrete need
 - We know what topics are important
 - We can run them with subtitles
 - We can run some in other languages

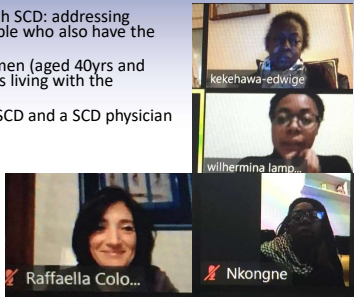



SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

12

Meet the expert patient session outcomes YOUNG PEOPLE

- Young people living with SCD: addressing questions to adult people who also have the condition
- Adults with SCD: 2 women (aged 40yrs and 60yrs) who are mothers living with the condition
- A teenager living with SCD and a SCD physician as moderators



European Reference Network
ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

13

Meet the expert patient session outcomes (7 questions)

What are the 3 things you would change in your life going back?

« I would do more sports. From the beginning of my SCD follow up pediatricians always tell me not to do sport, that it is dangerous, that it is a risk for your joints. But sport practice is efficient and complementary to SCD treatments.»

« I would have done the bone marrow transplant, now it's too late»

« I would have chosen a better job, for finding a balance between profession and private life »

What is the most important problem being an ageing adult?

«FATIGUE and finding ways to cope with it »

What about mental health?

«Being a child with sickle cell disease is so difficult on your mental health, you always feel like a burden»

«We need better access to tailored mental health services for SCD– it's important»

« Teenager and children know that their parents also need help in dealing with the condition »

European Reference Network
ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

14

Other questions....


- Do you have to take medications your entire life?
- Pregnancy (Can I become pregnant? How was your pregnancy?)
- Hope
- Socializing, relationships
- New drugs
- Acceptance by society
- Stigma

European Reference Network
ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

15

As someone living with SCD when you have a painful crisis what do you want your doctor to ask you?

- Your doctor should EXPLORE with you what you need, and together agree on treatments
- Your doctor should LISTEN to you and your partner/parent!
- Your doctor should BELIEVE you
- Your doctor should FOLLOW your lead
- Your doctor should ASK how this will affect your work



When the doctor asks what I need, it's a quick admission. When they don't listen, I know I will end up here for days...

European Reference Network
ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

16

What feature in any new therapy would make you agree to adopt it?

- The RISKS are more important to me than the BENEFITS
- What do I have to LOSE rather than GAIN?
- Will there be any permanent damage?
- How invasive will it be?



How will this fit into the rest of my life- work, family, activities?

European Reference Network
ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

17

What better ways are there for your doctor to communicate with you?

- No time limit
- Honest conversation
- Put me in touch with other people on the same medication

What is the right term- patient? Person living with sickle cell disease?


- Person!!! Not patient!
- Ban the term "sickler"
- "disease" makes people worry about contagion

European Reference Network
ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

18

How do you tackle racism and stigma affecting your care?

- People don't know about SCD or they don't care
- Policies would need to change to reduce racism and inequalities
- Access to care in Africa and India is also a massive problem due to stigmatization
- There are lots of assumptions about skin colour and requirements for testing
- We need to educate society, especially medical students and junior doctors
- Systemic racism in UK means prescription charges not free like in other chronic health conditions



Can we produce some educational material together?

European Reference Network
ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

19

SCD: My life, My words

Testimonies from pediatric patient and parents of people affected from different countries (France, India, Canada, UK).

UK patient- 9 years old "SCD is Hard but manageable"

Canada- Unexpected diagnosis; Fear and expectations
Answering questions 'why me?' and dealing with emotional consequences of dealing with limitations, pain and setbacks

Africa
Focus on the positives- you can live a normal life
We need more research to stop pain
Lots of new treatments coming along

India
Lack of education
Effect on whole family
Burden on whole population as well (e.g. birth controls??)
Living in remote areas means poor access to treatment

European Reference Network
ASCAT
SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

20

We leave you with our final key messages

- We need to improve access to care in Africa and India. It is OUR responsibility- all of us
- We have a life outside hospital!
- Global Awareness on SCD and research availability on SCD
- Racism and stigma affects our health everywhere and all the time. What will you do about it?
- How do we improve psychological and mental wellbeing?
- Please LISTEN to us

European Reference Network logo and ASCAT logo are present at the bottom.

SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online

21

Thanks to Participants and Coordinators of the SCD Patient Educational Meeting at ASCAT 2020

Ascat Steering Committee
BSH
ERN-EuroBloodNet

Sessions and videos WILL BE UPLOADED

European Reference Network logo and ASCAT logo are present at the bottom.

SCD people affected Educational Session at ASCAT 2020, 28th October 2020, Online


22

Feedback on ASCAT patient sessions 2020

18 participants

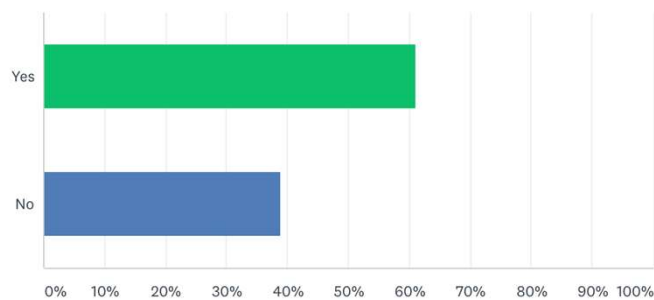
1

Q1

 Customize  Save as ▼

Was this your first time at ASCAT?

Answered: 18 Skipped: 0



ANSWER CHOICES	RESPONSES	
▼ Yes	61.11%	11
▼ No	38.89%	7
TOTAL		18

2

Q2



Customize

Save as ▾

What is your overall feedback on the 3 days?

Answered: 17 Skipped: 1

4.5★
average rating



	1	2	3	4	5	TOTAL	WEIGHTED AVERAGE
★	0.00% 0	0.00% 0	5.88% 1	41.18% 7	52.94% 9	17	4.47

3

Q3



Customize

Save as ▾

What did you think of the way things were organised (emails, contacts, etc)

Answered: 18 Skipped: 0

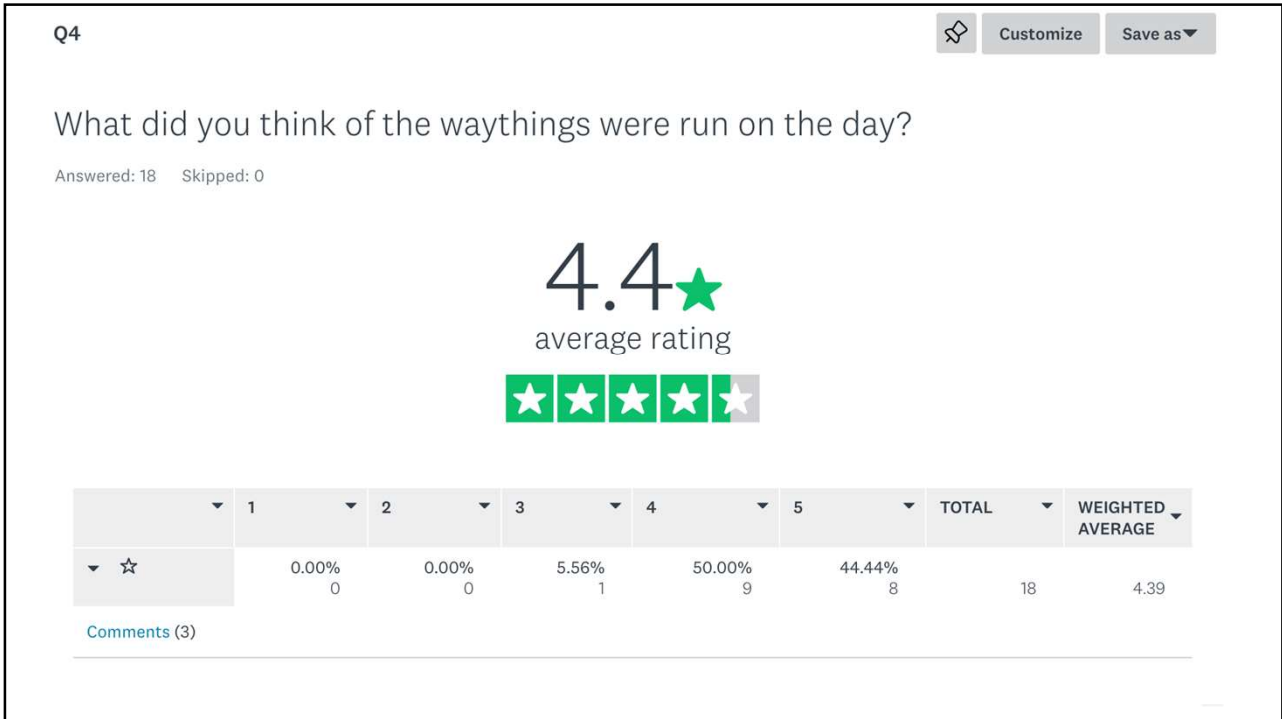
4.3★
average rating



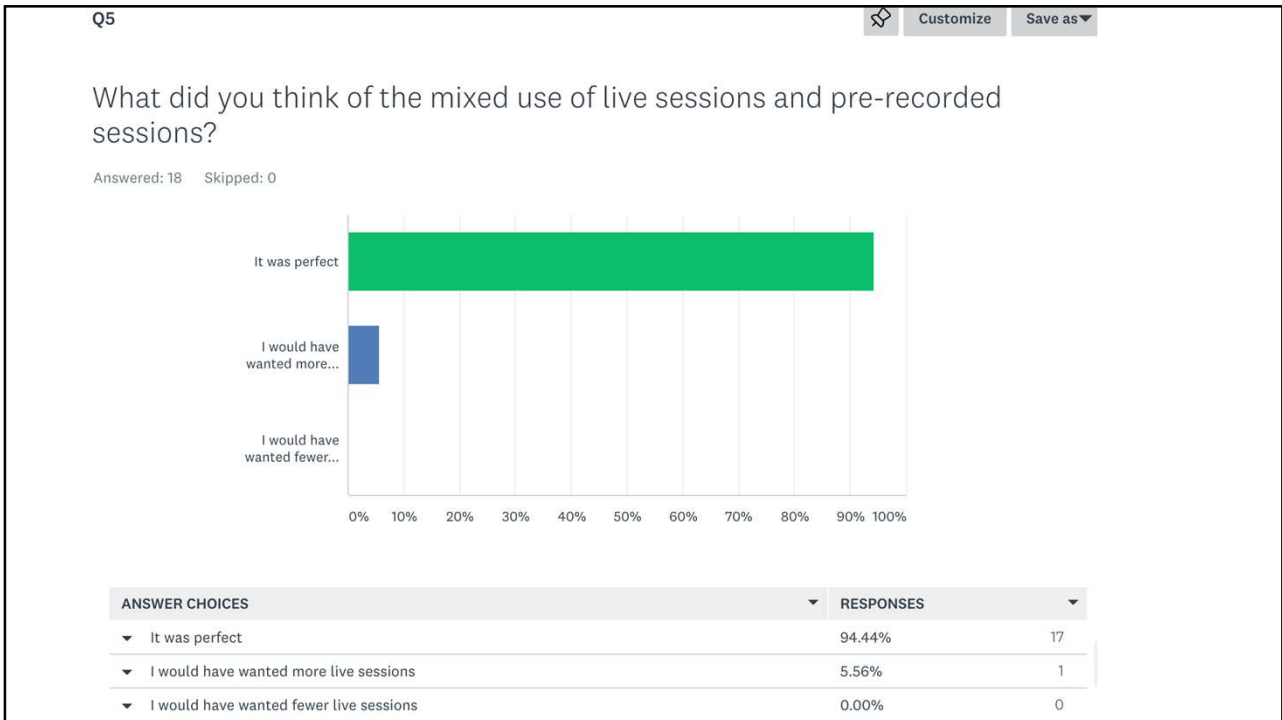
	1	2	3	4	5	TOTAL	WEIGHTED AVERAGE
★	0.00% 0	0.00% 0	11.11% 2	44.44% 8	44.44% 8	18	4.33

[Comments \(2\)](#)

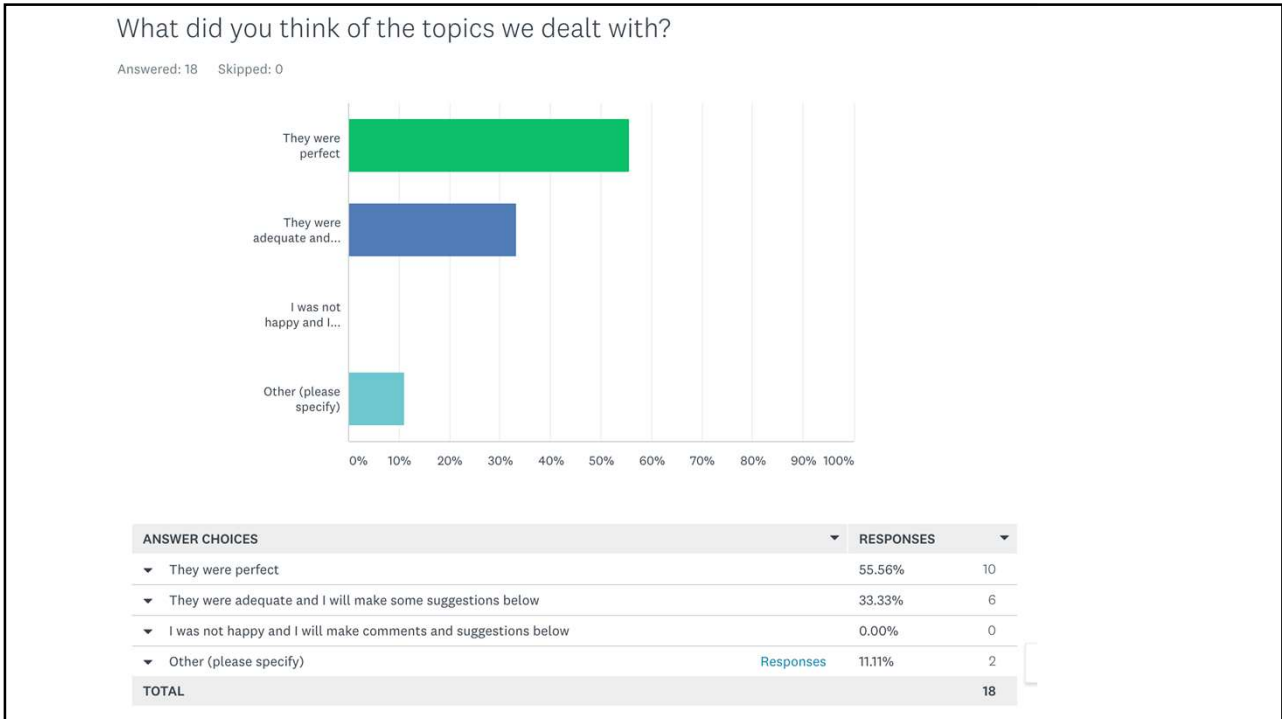
4



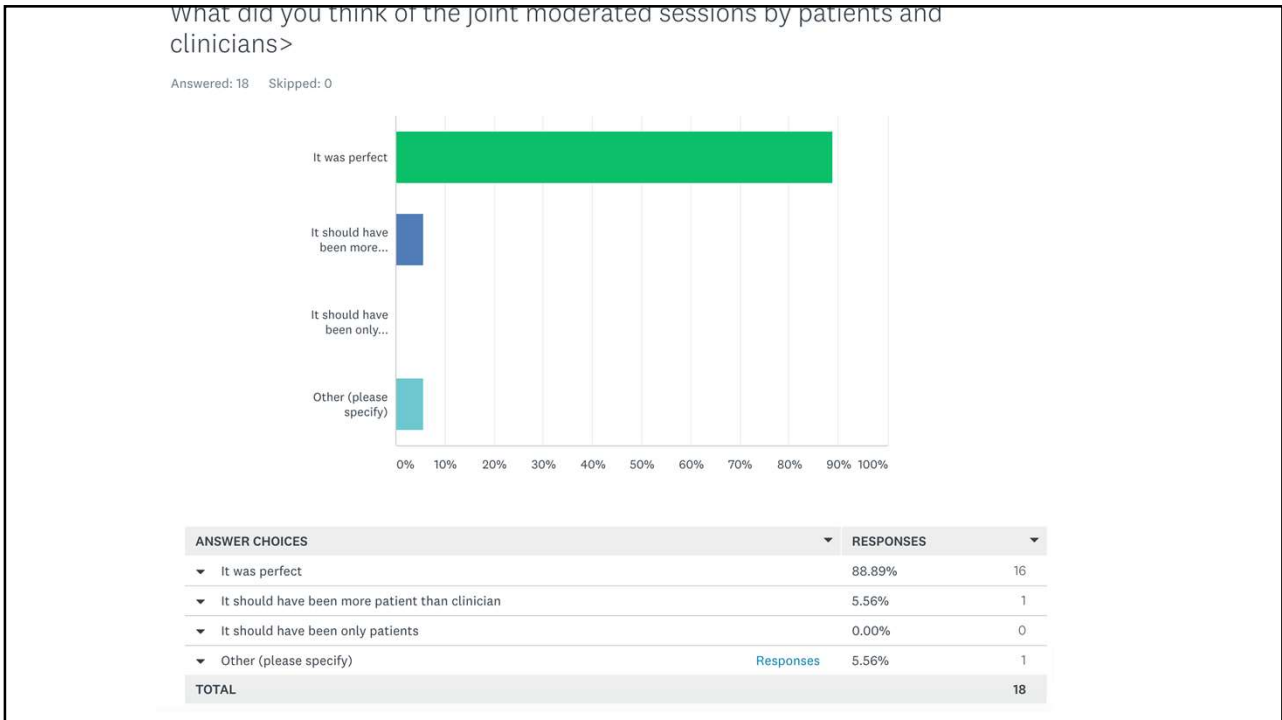
5



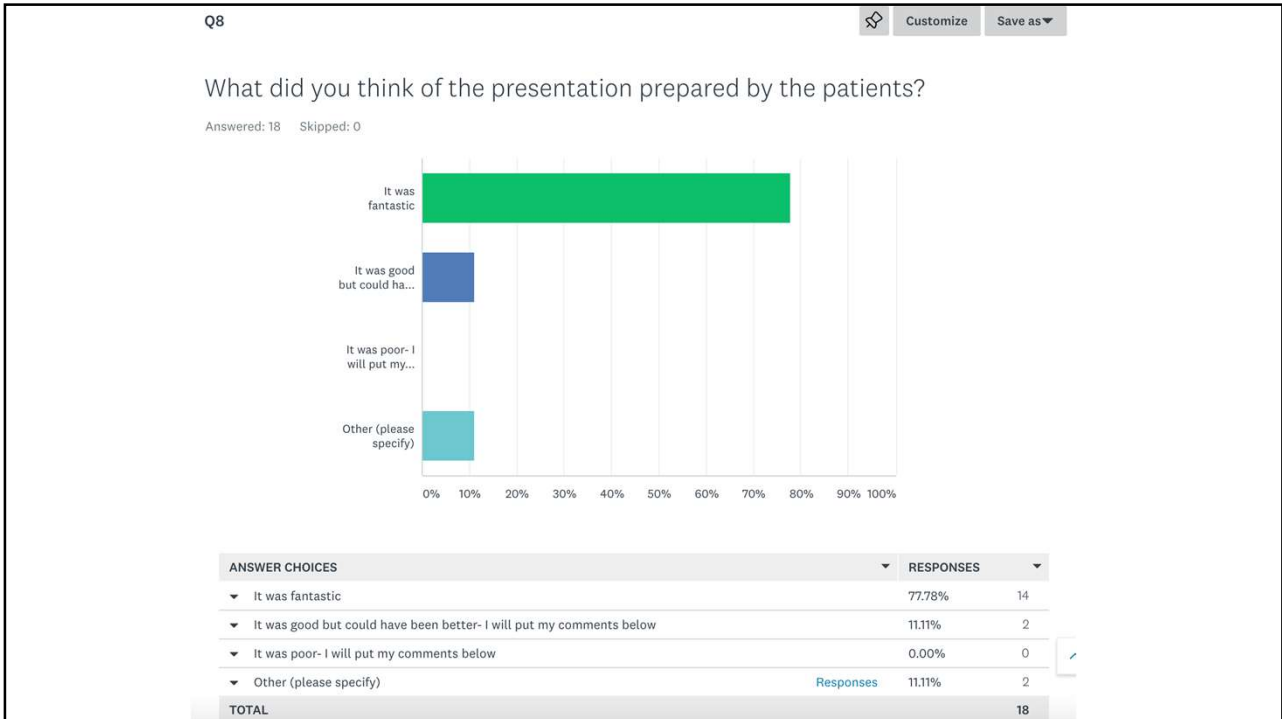
6



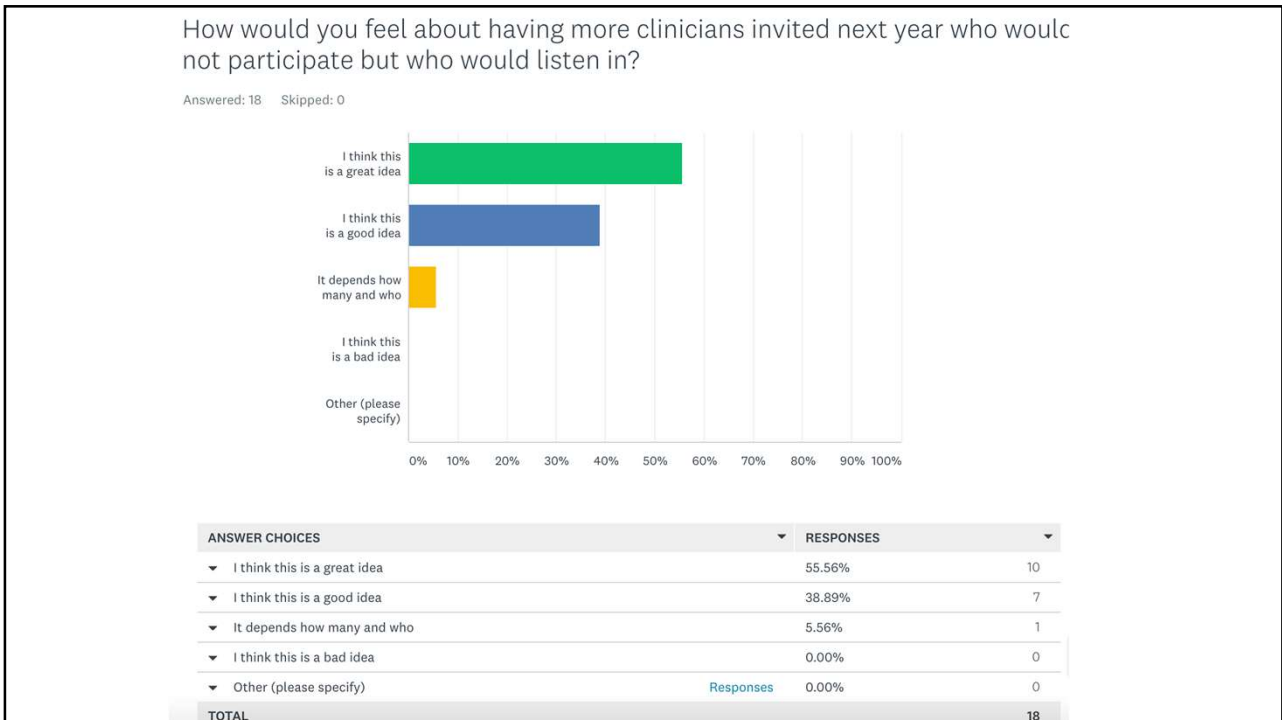
7



8



9



10

Q10 Save as ▼

Any other comments? Tell us what you liked most, what you liked least, what we should do differently next year? Was it too short? Too long?

Answered: 13 Skipped: 5

RESPONSES (13) WORD CLOUD TAGS (0) Sentiments: OFF

PAID FEATURE
Text Analysis lets you search and tag comments and see word clouds of frequent words and phrases. To get this feature, upgrade to a paid plan. ✕

[UPGRADE](#) [Learn more »](#)

Apply to selected ▼ Filter by tag ▼

Showing 13 responses

All were absolutely fine
12/9/2020 10:06 AM [View respondent's answers](#) [Add tags ▼](#)

It will be great if the conference were a litte bit translated.
12/8/2020 7:26 PM [View respondent's answers](#) [Add tags ▼](#)

11

Showing 13 responses

I totally enjoyed every session and currently looking forward to the next ASCAT.
12/8/2020 4:05 PM [View respondent's answers](#) [Add tags ▼](#)

From the beginning we were told it would be after working hours and that was not the case. It was difficult to attend any sessions before 17:00 GMT. It would be better to have sessions every day but less time each day.
12/7/2020 8:44 PM [View respondent's answers](#) [Add tags ▼](#)

Weekday sessions started at 5pm UK time which is still working time for most. Difficult to make some sessions. Other than that just not enough time, it flew by
12/4/2020 9:39 AM [View respondent's answers](#) [Add tags ▼](#)

I would like to give more time and more topics for discussions sessions between patients-parents / adults patients-young patients / patients-clinicians
12/3/2020 9:06 PM [View respondent's answers](#) [Add tags ▼](#)

12

Showing 13 responses

- Betting more in patient opinions
 12/2/2020 10:34 PM [View respondent's answers](#) [Add tags](#) ▼
- THANK YOU - I APPRECIATE ALL OF THE HARD WORK IN MAKING THIS HAPPEN. Please ensure the questions raised, and outcomes agreed are actioned and answered, and continual feedback and updates are received.
 11/30/2020 8:23 PM [View respondent's answers](#) [Add tags](#) ▼
- Perfect opportunity for patients. Please invite more from all parts of the world
 11/29/2020 1:15 PM [View respondent's answers](#) [Add tags](#) ▼
- patients involvement - participation and the way that good medical practice from doctors have met patients needs
 11/29/2020 11:26 AM [View respondent's answers](#) [Add tags](#) ▼

13

- I enjoyed it but it was obviously limited to an online experience due to the corona virus. I do hope that next year we will be able to meet face to face again. Many thanks.
 11/27/2020 8:44 AM [View respondent's answers](#) [Add tags](#) ▼
- Great organization. Thanks a lot, I really enjoyed this conference.
 11/26/2020 8:17 PM [View respondent's answers](#) [Add tags](#) ▼
- A session to prep both patient and physician moderators on roles beforehand, better clarity to be provided to patients to record testimonials on topic and audio visual quality, greater attendance of physicians to listen in,
 11/26/2020 5:26 PM [View respondent's answers](#) [Add tags](#) ▼

14

ANNEX II

ASCAT 2022

DELIVERABLE 6.5 ERN-EUROBLOODNET REPORT ON ACTIONS
FOR THE PROMOTION OF PATIENTS' EMPOWERMENT 2



European
Reference
Network

for rare or low prevalence
complex diseases

 **Network**
Hematological
Diseases (ERN EuroBloodNet)

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

More info on the full ASCAT program: <http://www.scorecharity.com/>

Wednesday 26th of January 2022

2 pm – 4 pm (UK Time)

3 pm – 5 pm (Paris time)

SCD Patients Educational Session by ERN-EuroBloodNet&ASCAT: Spotlight on Racism

An interactive webinar lead by expert physicians. Open discussion with the audience.

Moderators: **Mariane de Montalembert**, SCD expert physician, *AP-HP Hôpital Necker*, **Awa Kekeh**, patient representative.

Speakers: **Noémi Roy**, SCD expert physician, *Oxford University Hospitals, NHS Trust* & **Zeshan Quershi**, *University of Cambridge*, paediatrician

5 pm- 7. pm (UK Time)

6 pm – 8 pm (Paris Time)

SCD Patients Educational Session by ERN-EuroBloodNet&ASCAT: Spotlight on Mental Health

An interactive webinar with patients testimonies and lead by two specialised psychologists. Open discussion with the audience

Moderators: **Raffaella Colombatti**, SCD expert physician, *AUO Padova, Italy* and **Mary Akua Ampomah**, Patient advocate.

Speakers: **Gabriela Medin**, Psychologist and Psychoanalyst, *Hospital General Universitario Gregorio Marañón. Madrid* & **Adrienne Lerner** Clinical Psychologist, Psychologue clinicienne, *hôpital Louis-Mourier, Colombes.*

Testimonies: **Biba Tinga** and **Decio Témporario**, Patients representatives

Thursday 27th of January 2022

2.30 pm – 4.30 pm (UK time)

3.30 pm – 5.30 pm (Paris time)

SCD Patients Educational Session by ERN-EuroBloodNet&ASCAT : Spotlight on Pregnancy

An interactive panel lead by an expert physician, a specialised obstetrician and a patients representatives. Open discussion with the audience

Moderators: **Celeste Bento**, SCD expert laboratory genetist, *Centro Hospitalar e Universitário de Coimbra, EPE*, **Cassandra Trimnel**, SCD patient representative

Pannelists: Prof **Oteng-Ntim Eugene**, Consultant Obstetrician (Guy's and St Thomas' NHS Foundation Trust), Professor of Obstetrics (King's College London) & **G. Eda Ömür**, SCD patient representative, Prof **Laure Joseph**, SCD expert physician (AP-HP Hospital Necker, Paris)

Friday the 28th of January 2022

2 pm – 4 pm (UK time)

3 pm – 5 pm (Paris time)

2.SCD Patients Educational Session by ERN-EuroBloodNet&ASCAT: Spotlight on Bone Marrow Transplant

An interactive panel lead by one expert physician and a patient representative. Open discussion with the audience

Moderators: **Subarna Chakravorty**, SCD expert physician, *King's College Hospital NHS Foundation Trust* and **Agnes Nsofwa**, SCD Patient representative

Pannelists: **Marco Zecca**, BMT physician, *Fondazione I.R.C.C.S. Policlinico "San Matteo", Pavia*. **Constant Voduhe**, SCD Patient representative, **Luce Sona**, SCD Patient representative.

Plenary Patients Feedback

7.45 pm - 8.10 pm (UK time)

8.45 – 9.10 pm (Paris time)

Participants to the ERN-EuroBloodNet&ASCAT SCD Patients Educational sessions presenting the feedback of the program to the plenary session of ASCAT 2022






ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022


- RACISM
• MENTAL HEALTH
- BONE MARROW TRANSPLANT
• PREGNANCY

January 20-22 Virtual Global Conference


SINCE 2019 PATIENTS ARE AT THE CORE OF ASCAT CONGRESS

- A wonderful group of clinicians from all around the world who support the meaningful involvement of patients worldwide
- A wonderful group of people living all around the world with SCD who support clinicians to better understand patient needs and viewpoints
- Capacity to improve information, build collaborations, influence research and impact on care models
- Presentation of the results at the Plenary audience of ASCAT by SCD Patient Representatives



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

ASCAT 2019 PATIENT SESSIONS OUTCOME




SCD TOP TEN RESEARCH TOPICS

Lived experience and the SCD research agenda


OUTCOMES OF A PRIORITY SETTING WORKSHOP WITH SCD PATIENTS AND CAREERS, ASCAT 2019

- 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? How does staff diversity or lack of diversity affect the way patients experience racism, discrimination and stigma?
- 5 How do we harness the political agenda to improve care for sickle cell disease?
- 6 How can we optimise pregnancy for mother and baby in sickle cell disease?
- 7 What complementary therapies work in sickle cell disease including CBD oil and cannabis?
- 8 How do we manage chronic pain?
- 9 What is the impact of a comprehensive care team on outcomes for people living with sickle cell disease?
- 10




ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022


ASCAT 2020 PATIENT SESSIONS AND OUTCOME



THE VIRTUAL CONFERENCE FORMAT BROUGHT MORE PEOPLE TOGETHER

- COVID session - SCD patients experience during lockdown
- Peer Review process and participation of SCD patients in research studies
- Meet the medical experts
- Meet the expert patients
- SCD: My life, my words



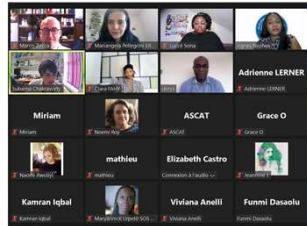


ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

THIS YEAR WE ARE STILL SADLY NOT FACE TO FACE... BUT WE REMAIN CONNECTED!



- We have shared our personal experiences
- We are continuing to build partnerships across countries
- We share good practices and ideas for patient support groups
- We strengthen the dialogue among patients and between patients and clinicians



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Key features of the patient sessions at ASCAT



- Moderators include healthcare professionals *and patient representatives*
- Topics were chosen by participants of last ASCAT edition
- Speakers are both health professional *and patient representatives*
- All patients who participated had opportunity to ask questions in chat and/or live

PATIENTS ARE EXPERTS!



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

NUMBER OF PARTICIPANTS AND DEMOGRAPHY



- 54 people registered to the only event: 34 people living with SCD and 20 health professionals or other role, mostly from European Country

...+ an average of 50 people connecting from ASCAT Congress Platform

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

NUMBER OF PARTICIPANTS AND DEMOGRAPHY



We thought it could be a lack of opportunities for participants to openly express themselves

... however the interaction this year has been great and very productive (more clinicians)

→ Patients session advertised in the conference agenda


ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022



1st session RACISM





ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022




Spotlight on Racism

History of Racism and Sickle Cell Disease

- The history of sickle cell disease is connected with the history of anti-Black racism
- In the past, Healthcare professionals have contributed to racist sentiment
- We want to change this narrative !




ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022




Spotlight on Racism

RACISM IS DIFFERENT FROM **DISCRIMINATION**
...But both could be part of SCD personal journey


<p>- Skin colours</p> <p>If you have white or light-coloured skin and physicians do not believe you are affected by SCD</p> <p>If you have black or dark-coloured skin and you receive poor /worse treatment (i.e. Anti-Black racism)</p> <p>IT AFFECTS INDIVIDUALS but also fewer FUNDING for RESEARCH and ACCESS to RESOURCES</p>	<p>- Disease related</p> <p>Many health care providers see patients as - high flyers - drug addicts</p> <p>If you - look good - or put on a brave face, healthcare providers say you are faking and not in so much pain (gender bias)</p> <p>Invisibility of symptoms leads to delayed or no treatment</p>
---	--



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022




WHAT ARE THE OBSTACLES FOR HAVING DISCUSSIONS ABOUT RACISM? - patient poll results




ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Racism




- **TRANSITION:** In pediatric services you experience less racism / more empathy
 - ➔ Fear about what will happen when patients move to adult care
 - ➔ More funding for pediatric care
- Difficult to trust health care providers when you have experienced racism from them
- Delayed hospitalisations when you have experienced racism in the hospitals
- Feeling invisible and different



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022


Spotlight on Racism



~~RACISM~~ IS DIFFERENT FROM ~~DISCRIMINATION~~


...But both could be part of SCD personal journey

INCREASING THE **AWARENESS** OF THE
CONDITION IN THE PUBLIC AT LARGE WOULD
DECREASE RACISM & DISCRIMINATION




ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Actions






COMMUNICATION GROUP	MEDICAL GROUP	COMMUNITY GROUP
Spread results of workshop widely	Med schools: more SCD in curriculum	Sharing stories
Wider discussion about pain and response to pain	Med schools: specific racism modules	Providing peer support
Sickle cell "disease" vs "disorder"	Policy statements from Royal Colleges	Offer workshop to other patient groups in UK



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022



2nd SESSION MENTAL HEALTH

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Mental Health

**THERE IS NO HEALTH....
WITHOUT MENTAL HEALTH!**



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Mental Health

Current situation:

Psychological support services are not always available

- Not available in the hospitals
- Not considered in national health programs
- Stigmatization and cultural reticence

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022





Spotlight on Mental Health

Psychological Challenges of Living with SCD in pediatric age

Sickle Cell Disease is Invisible to Others

Numerous and **unpredictable** clinical manifestations often from first months of life

The crucial moment –announcement of the diagnosis
The child participates in the consultation, hears the diagnosis and is sensitive to parents' emotions









ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Mental Health

Psychological Challenges of Living with SCD at every age

- Deal with limitations and constraints (Adjusting to the condition)
- Speaking with others about the illness
- Body image
- Self-confidence
- Feeling different
- Projecting yourself in the future

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Mental Health



How to approach your Mental Health?

- There is not a one size fits all, journey should fit each patient or caregiver.
- Each one must do his/her own process of finding his/her own way to deal with this chronic disease
- Process requires time and an adequate follow up

- Different mental healthcare pathways for patients and for caregivers!



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Mental Health



How can psychologists help patients & their families?

- Psychologists can translate patients' and caregivers' emotions into words → liberating process that improve quality of life
- Psychologists should encourage personal growth and autonomy
- Psychologists can help the acceptance of the chronic condition

→ Psychologist can give you the tools to support your mental health



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Mental Health



What could be done?

- Multidisciplinary approach: **Physician ↔ Psychologist**: Learn one from the other
- **Service available** in the hospitals or in medical centers, in order to normalise this support within healthcare.
- Make public at large **aware of the importance of mental health**, fighting the stigma that needing psychological care means being « ill » or « weak »
- Creating more **awareness** on the role of psychologists (patients associations, HCPs, psychologists, public at large).



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

3rd SESSION PREGNANCY



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022



Spotlight on Pregnancy

WILL I BE A MOTHER IN THE FUTURE?

- Many children/teenagers affected by SCD are often told by physician they could not face motherhood
- Most children/teenagers affected by SCD are told by physician they will face an exceptionally high risk pregnancy

Children & their families grown up with the idea they could not face serenely pregnancy or they could not have children

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Pregnancy



WILL I BE A MOTHER ?

- Many women face the difficulty to check if the partner is a carrier or not
- Some women are scared of being pregnant

→ Women feel like they have to choose between their body safety and being a mum
→ Women feel like they have to choose between having a 'non-Hemoglobinopathy carrier' partner or having a baby

WISH TO BE JUST AS EVERY OTHER WOMAN

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022



Spotlight on Pregnancy

AND THE CONTRACEPTION ?

- Sometimes there is an inadequate knowledge on contraception
- During consultation with teenagers is difficult to face the topic having also the parents in the same clinic rooms (experiences reported both by physicians and patients)

→ Contraception should be part of the family plan!

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022



Spotlight on Pregnancy

AND DURING PREGNANCY?

- Lack of confidence in the medical team (do they know what is the best for my child?)
- Lack of communication with the clinical team, with other SCD pregnant women or mothers
- Lack of national (updated) guidelines adapted to each country and reality

→ Clinical protocol should be discussed together

ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Spotlight on Pregnancy



What could be improved?

- Raising awareness among health professional → do not deliver the message that pregnancy is impossible
- Personal tailored care
- Prevention, screening and full tested: being well prepared before starting a pregnancy journey
- Improving the dialogue "patient ← → health physician" : talking about the protocols and health management together. Discuss about best practices
- Co-management of patients between obstetricians and hematologist
- Raising communication between patients (share of experiences)
- More information - Community/Support groups with obstetricians, hematologist, nurses, psychologists...
- Education on carrier screening and options for couples at risk



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

4th SESSION BONE MARROW TRANSPLANT



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Allogeneic haematopoietic stem cell transplantation procedure



NOT AN EASY CHOICE!!!

- The only cure but not everyone is eligible
- Feeling guilty (survivor's guilt)
- Changing identity: who am I without pain? Going from someone who is "sick" to someone who is not
- Adequate Aftercare should be provided
- Eligibility bar is still very high



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Allogeneic haematopoietic stem cell transplantation procedure



NOT AN EASY CHOICE!!!

- Huge pressure on the parents who are responsible for the final decision of BMT.
- There is the need for shared information for every step and support from medical team.
- Parents and patients should receive the right preparation for making the choice.
- During the last 5 years the results have hugely improved



ERN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

Allogeneic haematopoietic stem cell transplantation procedure



NOT AN EASY CHOICE!!!

How to make it easier?

- Psychological support for parents is vital
- Patients should be carefully prepared to the transplant (No need to take a decision in hurry. Decision could be taken after months, this is very important for the outcome).



ENN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022

CONCLUSION



EDUCATION IS ALWAYS THE RIGHT WAY!

- AWARENESS FOR THE PUBLIC AT LARGE ON SICKLE CELL DISEASE
- CONTINUING MEDICAL EDUCATION FOR HEALTH PROFESSIONALS
- PATIENT THERAPEUTIC EDUCATION



ENN-EuroBloodNet&ASCAT SCD Patients Educational Session at ASCAT 2022



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

