6.4 ERN-EUROBLOODNET REPORT ON ACTIONS FOR THE PROMOTION OF PATIENTS' EMPOWERMENT

ERN-EuroBloodNet European Reference Network on Rare Hematological Diseases

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DOCUMENT INFORMATION

6.4 ERN-EuroBloodNet Report on Actions for the promotion of patients’ empowerment

Report Document

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

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Short Description

Report on activities implemented for the promotion of patients' empowerment including: mapping of patients’ associations, educational workshops and webinars, results from surveys conducted on patients’ expectations and quality of life, reporting of collaboration with EURORDIS and European Patients Organizations for the reinforce of patients’ advocacy sessions at International Congress.

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TABLE OF CONTENTS

1. INTRODUCTION

With the creation of the European Reference Networks (ERNs), the EU has provided the requirement and the means for empowering patients, raising awareness and promoting therapeutic patient education on rare diseases. In addition, ERN could provide cross border health assistance to Rare Hematological Diseases Patients, as ERN are structure for knowledge sharing and coordination of health care across the European Union.

When it comes to rare diseases, patients and patients' families could face isolation, because of the scarce access to information, lack of services and patients associations and finally sometimes a poor disease management. Because of this ground, the ERN-EuroBloodNet has started playing a key role in Europe for making rare diseases patients burdens visible to scientific community, public-at large and policymakers. The ERN-EuroBloodNet could achieve good goals in those domains thanks to the close collaboration with expert HCPs, EURORDIS, ePAGS and European, National or Local patient's representatives groups too.

ERN-EuroBloodNet strategy for improving the quality of life of patients living with rare diseases and fight patients isolations has been to invest a solid plan of patients' empowerment focusing on the mapping of disease geographical coverage of patients associations, promoting patient involvement in networks, investing in patients advocacy, and finally by improving health outcomes from the patient-centered approach.

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: to map Patients organisations across Europe and to identify ePAGs advocates. ERN-EuroBloodNet's aim is to facilitate patients righs 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 adressed 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 caregivers community:
    a. Educational patients' session at congress
    b. Videos testimonies
    c. Cross border health assistance
    d. Establishment of patients associations and advocacy training

2. Patients Advocates and Patients Organisations:
    a. Collaboration with ePAGs and Eurordis on comprehensive actions of the ERN-EuroBloodNet
    b. Topic on focus webinars

3. Both:
    a. Repository of Educational Material for patients
    b. European mapping of patients associations.
    c. Surveys

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

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2. PATIENTS ADVOCACY'S ACTIONS IMPLEMENTED

2.1 European Mapping of Patients Organizations and Increasing of European Patients Groups Representation for Hematology

Rationale

Patient organisations have a key role to play in healthcare. They help patients and their carers to understand their condition, to find out about how and where to get access to the best treatment and care, to learn how to live with the disease, its symptoms and side effects, and help them to navigate their healthcare systems to achieve best possible outcomes.

Patient organisations serve as a key contact point for patients and carers through local support groups, websites and social media. In addition, 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. To achieve those goals, patient organisations operate on a regional, national and international level today.

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.

Patients 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.

Objectives

Two main objectives have been defined to promote patients' advocacy:

a) to map patients associations existing in Europe for Rare Hematological Diseases and make them available on the ERN-EuroBloodNet website.

The exercise of mapping the associations has not only the aim of making RHD patients associations visible and joinable at European level. To have a comprehensive list of keys contacts of associations' means also receiving help in gathering evidence on patients' expectations and needs, mapping barriers to access of care and diagnosis and identifying where CBH intervention is needed. Finally the repository will not only lead to promote the findability of the associations, but also to the identification of areas lacking of patients' groups and which creation can be promoted in the core of ERN-EuroBloodNet.

b) to increase the European patients' groups representation for Hematology according to pre-identified gaps in the representation of not covered clinical areas

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.

Methods

European mapping of patients associations

The first mapping of patients associations has been performed based on the creation of a repository on the Associations listed on the eleven pan European Rare Hemagological disease Patients Network.

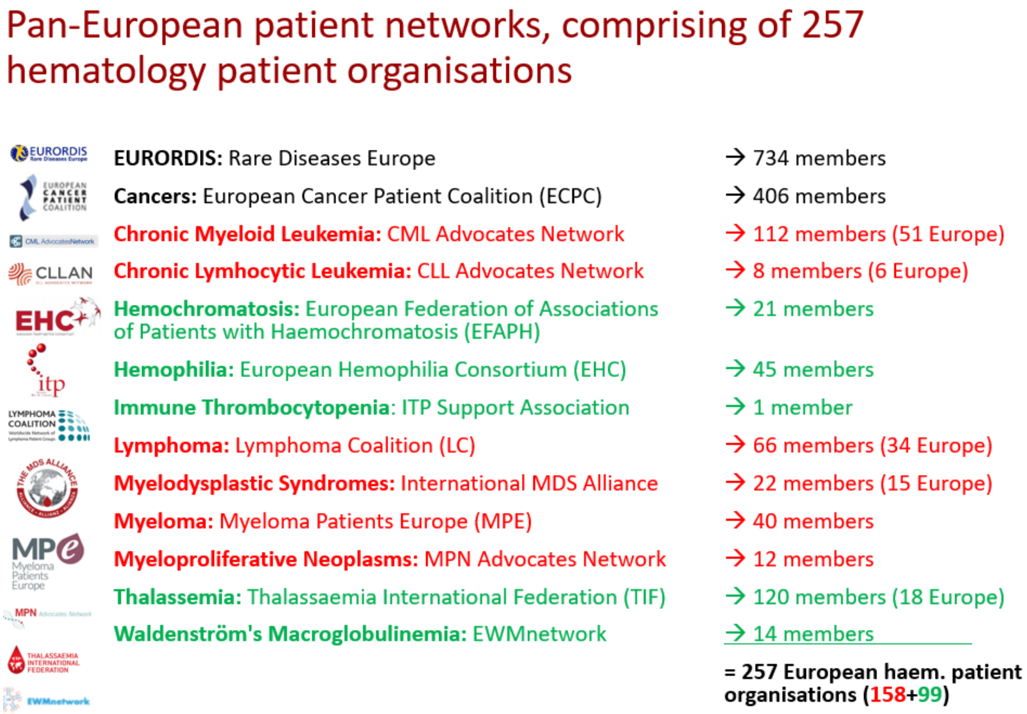
Increase of ePAGs for Hematology

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. It consisted of two mains steps: identifying those clinical areas not yet covered by an European patient representative and secondarily identifying potential candidates.

Results

European mapping of patients associations

Eleven pan-European umbrella networks 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.



Pan-European patient networks, comprising of 257 hematology patient organisations

EURORDIS: Rare Diseases Europe	→ 734 members
Cancers: European Cancer Patient Coalition (ECPC)	→ 406 members
Chronic Myeloid Leukemia: CML Advocates Network	→ 112 members (51 Europe)
Chronic Lymhocytic Leukemia: CLL Advocates Network	→ 8 members (6 Europe)
Hemochromatosis: European Federation of Associations of Patients with Haemochromatosis (EFAPH)	→ 21 members
Hemophilia: European Hemophilia Consortium (EHC)	→ 45 members
Immune Thrombocytopenia: ITP Support Association	→ 1 member
Lymphoma: Lymphoma Coalition (LC)	→ 66 members (34 Europe)
Myelodysplastic Syndromes: International MDS Alliance	→ 22 members (15 Europe)
Myeloma: Myeloma Patients Europe (MPE)	→ 40 members
Myeloproliferative Neoplasms: MPN Advocates Network	→ 12 members
Thalassemia: Thalassaemia International Federation (TIF)	→ 120 members (18 Europe)
Waldenström's Macroglobulinemia: EWMnetwork	→ 14 members
	= 257 European haem. patient organisations (158+99)

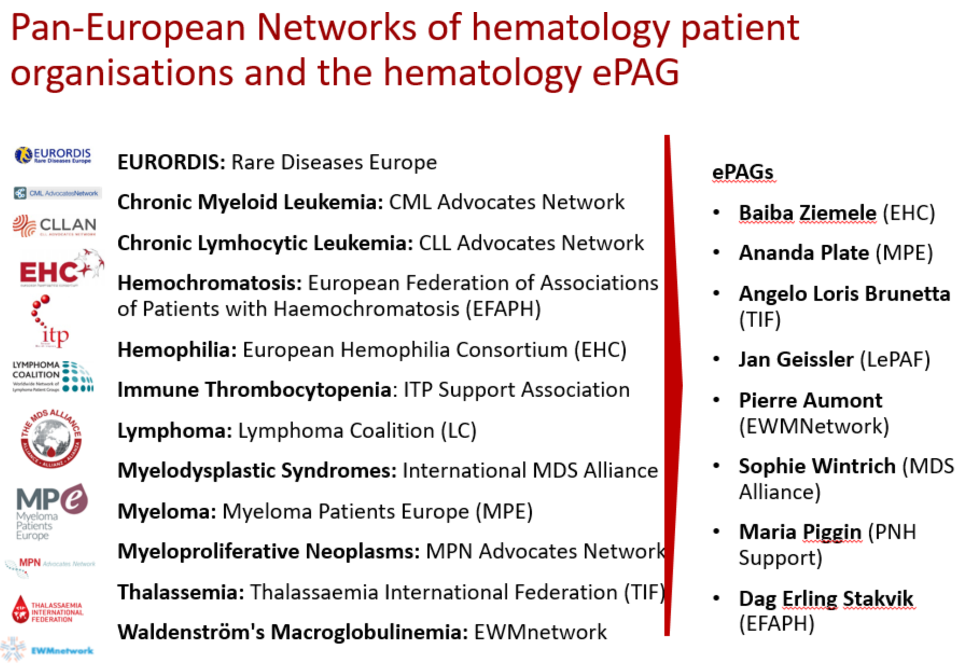
Fig.1 The 11 Pan-European RHD Patients Network listed on the ERN-EuroBloodNet Website. Networks that figure with dark ink are those that gathers transversally more than one ERN-EuroBloodNet subnetwork: EURORDIS covers all RDs, ECPC covers both lymphoid and myeloid malignancies. The organisations that are marked with red Inks are those that covers oncological rare hematological diseases, and those marked with green ink are associations that belongs to non-oncological ERN-EuroBloodNet hub. Finally, on the right: the counting of membership group affiliated to each organisation.

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. On the ERN-EuroBloodNet webpage (https://eurobloodnet.eu/patientsadvocacy/patient-advocacy-organisations), those Pan-European organisations are listed and by clicking them the web user can be addressed to the official organisation's webpage.

Increase of ePAGs for Hematology

The following new ePAGs have been designated:

- Several gaps have been identified regarding patients' representation in the network, as the areas of Sickle cell disease (SCD), Pyruvate Kinase Deficiency (PKD), and Hereditary Hemochromatosis and Iron metabolism related disorders subnetwork.
    - Mr Dag Erling Stakvik from the European Federation of Associations of Patients with Haemochromatosis (EFAPH) has been designated as ePAG for Hereditary Hemochromatosis and Iron metabolism related disorders subnetwork.
    - One patient for PKD has been identified and already been trained by EURORDIS. PKD representative should 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 2.2.
- Ms Baiba Ziemele from Euopean Hemophilia Consortium (EHC) has been designate as the substitute of Ms Amanda Box, previous ePAGs in representation of Bleeding Coagulation Disorders.



Pan-European Networks of hematology patient organisations and the hematology ePAG

EURORDIS: Rare Diseases Europe

Chronic Myeloid Leukemia: CML Advocates Network

Chronic Lymhocytic Leukemia: CLL Advocates Network

Hemochromatosis: European Federation of Associations of Patients with Haemochromatosis (EFAPH)

Hemophilia: European Hemophilia Consortium (EHC)

Immune Thrombocytopenia: ITP Support Association

Lymphoma: Lymphoma Coalition (LC)

Myelodysplastic Syndromes: International MDS Alliance

Myeloma: Myeloma Patients Europe (MPE)

Myeloproliferative Neoplasms: MPN Advocates Network

Thalassemia: Thalassaemia International Federation (TIF)

Waldenström's Macroglobulinemia: EWMnetwork

ePAGs

- **Baiba Ziemele** (EHC)
- **Ananda Plate** (MPE)
- **Angelo Loris Brunetta** (TIF)
- **Jan Geissler** (LePAF)
- **Pierre Aumont** (EWMNetwork)
- **Sophie Wintrich** (MDS Alliance)
- **Maria Piggin** (PNH Support)
- **Dag Erling Stakvik** (EFAPH)

Fig 2. In the figure are listed the Pan-European Network of Hematology Patients Organisations and the 8 ePAGs affiliated to the ERN-EuroBloodNet.

Next steps

The following steps are foreseen for the mapping exercise:

- Completing the mapping of patients associations based on the implementation of patients associations profiles at the website
- Adding the following Patients Association Search Tool to the ERN-EuroBloodNet website.
    - 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/

On the other hand, the designation of an ePAG for PKD and SCD will be finalized.

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

Rationale

Up to now, Sickle Cell Disease (SCD) does not have in every country in Europe an adequate educational contribution for patient's awareness on daily management of the disease. SCD is indeed traditionally endemic in African and Middle East countries but their frequency has increased recently in Europe due to migration and mobility flows. 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 has identified the urgent need of having a solid representation of SCD in Europe. In many European Countries there are no patients association covering SCD. As consequence, in Europe SCD patients and their families suffer from isolation. A lack of patients' organizations means: no advocacy projects for SCD, no accessible information for patients, no psychological and human support offered to patients by meeting other patients. 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 planned to establish the European Network of Sickle Cell Disease Patients Organisations.

Objectives

The ERN-EuroBloodNet together with EURORDIS by establishing the European Network of Sickle Cell Disease Patients' Organisations aims 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.

Methods

First step overtaken by the task force has been to define the characteristic of the European Network of SCD Patients' Organisations:

- A bottom-up umbrella network of national and local organizations of SCD patients' advocates
- Each Country is represented by two spokespersons. A spokesperson is a national representative that advocate on behalf of all the national SCD patients' organizations. The role is the mediation among the Euroepan Netwotk of SCD Patients' Organisations and the national patients support groups.
- Among all spokespersons, a representative one will become ePAG for the ERN-EuroBloodNet.

Second step overtaken by the task force has been to define how to plan the action itself of the establishment of an European Network of SCD Patients' Organisations.

1. Identify SCD Patients support groups existing in Europe.
    a. It has been consulted the Repository of Patients Association created by Orphanet and by ENERCA (the European Network of Rare and Congenital Anemia established before the ERN-EuroBloodNet).
    b. It has been asked to ERN-EuroBloodNet SCD experts and Red Blood Cell Expert to provide the contact of associations if existing in their country.
    c. If no support group exist, together with SCD expert members, it has been identified motivated patients, that is to say patients that would have like to operate as patients advocate.
2. Invite Patients support group and/or motivated patients to National Meetings. National Meeting are an occasion to present the project of the establishment of an European Network of SCD Patients organisations and to listen to SCD patients' needs and expectation.
3. Encourage, if not existent yet, to form a national association/federation/group
4. Countries reached by national meetings elect their national representatives.
5. First meeting where the national representatives elected will meet for planning chart of conduct and actions.
6. Organizing educational sessions for empowering patients advocates (for instance ASCAT 2019, ASCAT 2020)

Results

Advocacy representation

- More than 50 SCD patients association have been reached in Europe and are constantly in contact with the ERN-EuroBloodNet 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 belong to 12 European Countries: Spain, Portugal, Italy, France (France metropolitan and France Overseas), Cyprus, Belgium, Germany, Ireland, UK, Austria, Netherlands, Switzeland.
- Also Patients associations and patients motivated to contribute to the cause of SCD advocacy outside Europe have been reached. For instance, the Australian Sickle Cell Advocacy or the GASCDO, the Global Alliance of SCD organisations. Other countries reached are: USA, India, Ghana, Kenya, Abuja.

National meetings

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

- First national meeting in Milan, the 29th of March 2019

- Informative National Meeting in Bruxelles, the 19th of June 2019. ERN-EuroBloodNet presenting the project during the international SCD day in Belgium
- Second National Meeting, in Paris, the 29th of June 2019
- Third National Meeting, in Madrid, gathering Spanish and Portuguese patients, 18th February 2020

The following points, based on the meetings held between France, Italy, Spain, Portugal and Belgium are common points raised by those countries and would be translated into first concrete actions at European level:

- Recognition of Sickle Cell Disease as recognized disability in the National Table of Disabilities.
- Fight against Stigmatization.
- Fight against patient isolation.
- Improving best practices and patients' pathways.
- Awareness raising on the importance of receiving psychological help.
- Neonatal and prenatal screening programs.
- Improving the visibility of the disease during the International Sickle Cell Disease Awareness day.
- Repository of Educational Material for patients.

Find the Outcomes from the National Meetings in Annex I Outcomes from National Meeting

One of the relevant results to underline from the national meetings are the effects those had at the national level on local patients organisations:

- In France the election of National Representatives for the European Network of SCD Patients Organisations has strength the collaboration between the associations belonging to the France overseas and the France metropole; as for the associations belonging to Parisian territory and extra urbans territories
- In Italy the patients associations got to know each other and choose to collaborate together. They also have elected two national patients representatives for the European Network of SCD Patients Organisations.
- In Spain, no SCD patients associations existed. After the meeting held by the ERN-EuroBloodNet a National patients association was founded: the ASAFE 5 Asociación Española de Enfermedad Falciforme; Two national patients representatives have been elected for the European Network of SCD Patients Organisations.
- In Portugal new patients representatives joined the existing National patients association: the APPDH. Two national patients representatives have been elected for the European Network of SCD Patients Organisations.

Congress presentation

A poster describing the project has been presented at the European Congress on Rare Diseases 2020, on 14-15, May. The ECRD is considered as the largest, patient-led and centered rare diseases event in Europe and at the Global level. For the edition of 2020 the event was online and has gathered over 1500 registered participants convened from 57 countries. The Poster presented at the ECRD 2020 is in the Annex II_Poster ECRD EU NET SCD PO

Videos Patients Testimony

As explained in the introduction of this Deliverable the ERN-EuroBloodNet is playing a key role in Europe for making SCD burdens visible to scientific community, public-at large and policymakers. Main aim was indeed to spread patients' needs and expectation by giving to patients the possibility of expressing themselves and providing editing and recording solutions, media channels and logistics assistance. This is the reason why for the World Sickle Cell Disease Awareness Day 2020 (WSCD) two SCD patients' testimony videos have been published on the EuroBloodNet's YouTube Eucational 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.

- WSCD 2020 EuroBloodNet -The establishment of the European Network of SCD PO: 9 patients from Portugual, Italy, Spain, France have given testimony on the video.

Here follows some rates obtained from the YouTube EuroBloodNet EDU Channel:

Video Title	**Views**	**Likes**	**Number of displays**	**Watch time hours**
WSCD 2020 EuroBloodNet -The establishment of the European Network of SCD PO	278	10	328	10,2

Fig 3. In the table are reported the Interaction registered on the YouTube EuroBloodNet EDU Channel.

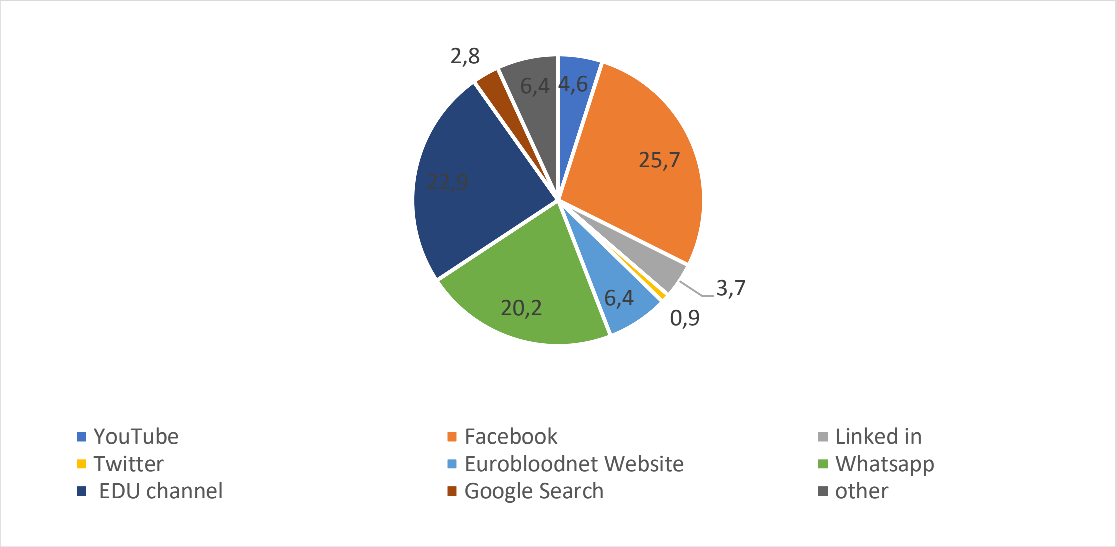


Fig. 4 Source of viewers traffic (%) of the video WSCD 2020 EuroBloodNet - The establishment of the European Network of SCD PO on YouTube EuroBloodNet EDU Channel.

Next steps

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

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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

Rational

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. Secondarily 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.

Results

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

The first case was registered in 2018: an adult patient suffering from Betha Thalassemie was moving from Italy to France (transfer of residence) and wanted to access to transfusion being reimbursed in the affiliation country as it was for the home country. Transfer of residence is not a reason of CBH assistance. Obstacles faces: the procedure of passing from one social security to another require time in which the patient had complications in accessing transfusion. Solution: SCD patients fount a job and automatically obtained the social security assistance.

The Second case in 2019: a Betha Thalassemia patients moving from Italy to France for a short-term job contract in France. Patient wanted to access to transfusion being reimbursed in the affiliation country as it was for the home country. But start a new job in an European Country is not a reason for CBH assistance. Obstacle faces: the procedure of 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 was helped with local administration and bureaucracy.

The third case is a result to be underlined as a successful example of Cross Border Health Assistance for Chronic preexisting conditions and programmed cares is the one that follows. In 2019 an italian student affected by thalassemia, came to France to follow an Erasmus study program and had to schedule cares such as transfusions and related treatments. No linguistic nor administrative mediation was guaranteed for the implementation of access to healthcare procedures. Patient family contacted the ERN-EuroBloodNet following a Patient Association's suggestion. The ERN reassured the patient, explaining he/she was entitled to assumption of costs and taking the role of mediation. French social security fund as well as the health facility required the provision of a S2 form by the insured person. The affiliation's home country fund refused to issue S2 form, since this insured person can be treated in his/her country within a time frame compatible with her state of health. So the ERN-EuroBloodNet contacted the French National Contact Point for Cross-Border Care (NCP Cleiss https://www.cleiss.fr/) and asked to play a key role among patient' home and host countries social security funds. French NCP Cleiss communicated with both Member States explaining S2 form was not the appropriate document to the situation of a patient studying in France in the framework of the Erasmus project. In light of European legislation (Regulations (EC) 883/2004 and 987/2009), patient is considered to be temporarily residing in France during his/her studies. Patient benefits from reimbursement of his/her health care costs by the French scheme (on behalf of the home health insurance fund) on presentation of his/her European health

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insurance card and in accordance with the legislation and reimbursement rates applicable in France. The European Health Insurance Card makes it possible not only to cover medically necessary care during a temporary stay outside one's habitual State of residence but also chronic or pre-existing illnesses insofar as the purpose of the stay is not to seek care. These measures are intended to ensure that the person may not have to return to his or her State of affiliation for care but may continue his or her stay in safe conditions. Thanks to the ERN-EuroBloodNet and French NCP Cleiss cooperation, the patient did not feel lost in the management of bureaucracy and was able to benefit from the temporary cross-border mobility of his/her healthcare rights according to the application of the above mentioned regulations and the provisions of Decision S3 taken by the Administrative Commission for the Coordination of Social Security Systems.

The fourth case is registered in 2019. A French SCD Parent' Patient contacted the ERN-EuroBloodNet for helping her daughter (SCD patients) studying abroad, in Madrid. The problem was that the parent patient felt her daughter was not receiving an adequate clinical patient management in Spain. The ERN give to SCD patient and family the availability to contact expert physicians in Spain but the patient did not answered back.

The fifth case is registered in 2019. The ERN-EuroBloodNet was contacted for finding expert centers in Scotland that could follow the patients' management of three SCD pediatric patients. The family was moving from France to Scotland. The ERN has contacted UK SCD experts and identified HCPs and physicians in Scotland. Moving abroad in not forseen in the framework of CBH Directive, but the ERN-EuroBloodNet provide cross border assistance in finding expertise abroad by cooperating with the members of the Network.

The sixth case is registered in 2019. A French physicians contacted the ERN-EuroBloodNet for asking for cross border assistance in finding a SCD expert that could follow the patients management of an adult patient moving to Spain. Moving abroad in not forseen in the framework of CBH Directive, but the ERN-EuroBloodNet provide cross border assistance in finding expertise abroad by cooperating with the members of the Network.

A seventh case is registered in 2020. An adult Italian patient contacted the ERN-EuroBloodNet for finding an expert center in UK, because the patient wanted to live for a short period in UK. This request has been stopped with the outbreak of the covid 19 pandemic.

An eight case is registered In 2020 .The ePAG represented PNH contacted the ERN-EuroBloodNet for giving assistance to a PNH Adult Patient from Bosnia (not EU MS) that 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).

Analysis

From the eight request received, three 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.
2. 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.
3. For many different conditions, transfusions need to be performed. In many country accessing to transfusions for a patients coming from anotherr euopean 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 are not clear and the patients feel lost in procedures.

Next steps

The possibility of adding a "Contact us" section on the ERN-EuroBloodNet website for assisting Cross Border Health requests will be evaluated.

3.2 SURVEY ON THE ACCESSIBILITY TO TRANSFUSIONS AND RELATED TREATMENTS, IF THE PATIENT IS ABROAD, IN A MEMBER STATE OF THE EUROPEAN UNION.

Rationale

The majority of cross border health assistance requests received within the ERN-EuroBloodNet were sent by patients for accessing transfusions in a European Member States. Considering this, the ERN-EuroBloodNet Coordination Team decided to launch a survey for evaluating the accessibility to transfusions in Europe in the framework of the Directive 2011/24/EU, Regulations (EC) 883/2004 and 987/2009.

Objective

To monitoring the cross border health accessibility to blood transfusions across Europe in the framework of the Directive 2011/24/EU, Regulations (EC) 883/2004 and 987/2009 by identifying studies case for the evaluation of obstacles or virtuous models.

Methods

The ERN-EuroBloodNet Coordination Team has first created the survey's items by defining which results would have been appropriate to gather through the questions.

The survey contains open questions with a free text for answering. A qualitative analysis of results is required, as there are not scores to apply.

- Are you... patient/caregiver?
- Patient's age
- Nationality
- Did you experience a transfusion and related treatment (i.e. chelation) abroad?
- What disease do you need transfusion and related treatment (i.e. chelation) for?
- To which country abroad did you go?
- In which country were you resident at the time of your stay abroad?
- When? If you don't remember the date, please specify at least the year:
- For how long did you stay abroad and need transfusion and/or related treatments (i.e. chelation)?
- Which where the reasons of your permanency abroad?
    - If previously ticked "other reasons" please explain
- Did you find any barriers in getting transfusions?
    - If you previously ticked "yes," could you please describe the obstacles you found?
- Did you find any barriers in getting related treatments (i.e. chelation)?
    - If you previously ticked "Yes" could you please describe the obstacles you found?
- Would you like to be contacted by us and be part of a study case?
    - If you previously ticked "Yes", please tell us how to contact you

For evaluating if the survey was properly developed a test has been conducted on three voluntaries patients. The survey has been reshaped following their advices and opinions. Then the survey has been translated into Italian, French, and Spanish. The survey was then managed via the EU Survey platform. Finally, the survey was disseminated through those patients associations belonging to clinical area in the scope of the ERN-EuroBloodNet for pathologies that would require blood transfusions.

Results

First answer: A SCD Patient, 35 years old and living In France. Patient moved abroad for professional reasons in Netherlands. Patient did a vaso-occlusive crisis and needed a transfusion. But in the urgency hospital where she was hospitalized did not perform it because of a lack of knowledge of the SCD.

Second answer: an adult patien, 36 years old and living in Italy, suffering from Betha Thalassemie was moving from Italy to France (transfer of residence) and wanted to access to transfusion being reimbursed in the affiliation country as it was for the home country. Obstacles faces: the procedure of passing from one social security to another require time in which the patient had complications in accessing transfusion.

Third answer: an adult patient, 54 years old, suffering from PNH wanted to go abroad but no transfusion were available. This answer is n/a as the patient declared also that no obstacle were found in requesting transfusion. The host country is not specified.

Fourth answer an adult patient, 31 years old, suffering from CDA did not experience the possibility of accessing to transfusions abroad.

Fifth answer: an adult patient, 43 years old, Italian, suffering from Beta Thalassemia Major, wanted to go to USA for a clinical trial did not find any obstacles in receiving transfusions.

Sixth answer: An adult patient of 20 years, coming from France and suffering from SCD studying abroad, in Madrid.
Find obstacles in accessing transfusion because was not receiving an adequate clinical patient management in Spain ("lack of blood follow up") and had not the European Health Insurance Card.

It is important to underline the three major obstacles in accessing transfusions

- no recognitions of the Directive 2011/24/EU or Regulations (EC) 883/2004 and 987/2009. in hospital administration
- no available transfusion
- physicians abroad did not have an adequate clinical knowledge of the disease

From those obstacle it results very important to integrate ERN at National Level

Next steps

Considering the scarce number of answers, but the good results of the analysis, it could be reconsidered the possibility of conduct again the survey after improving the dissemination strategy among HCPs, patients associations and scientific national societies. In addition, an analysis of the study cases could be launched, as all the patients answered that they would approve to analyze their experience as a study case.

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 and " 6.1 ERN-EuroBloodNet Report on Actions for the promotion of continuing medical education on RHD" 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
2. Repository of Educational Material for patients
3. Comprehensive webinar program for patients associations
4. Patients Testimony videos

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 RELATED ACTIONS

4.1.1 EDUCATIONAL PATIENTS SESSION AT CONGRESS

Rationale

ERN-EuroBloodNet is promoting educational activities taking place during International Scientific Congress and targeting people living with SCD 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. In addition, 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.

Finally, 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. 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.

SICKLE CELL DISEASE RESEARCH PRIORITIZATION WORKSHOP AT ASCAT 2019

Objective

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

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

Methods

Workshop was held the 20th of October 2019 following an adapted methodology from the James Lind Alliance to gain a valid consensus. The workshop was moderated by three experienced facilitators from Oxford University Hospitals: Dr Noémi Roy, Sandy Hayes and Dr Catriona Gilmour-Hamilton.

The first step was to create a patient forum with at least 20 patients coming from EU countries to share experiences, built cross-border partnerships and discuss good practice for local patient support groups. SCD patients were identified through ERN-EuroBloodNet SCD experts' members, National SCD Networks, ASCAT members and EHA ERN-EuroBloodNet representatives.

Patients were then asked to brainstorm what issues relating to their care they felt requires research focus. These ideas were then grouped into themes, and formulated into individual research questions. Patients then voted on the questions of greatest importance to them, generating an overall "Top 10" research questions chosen by the patient community. The brainstorming and theme grouping led to the formulation of 42 individual research questions. Patients were then given a hypothetical "10 million dollars" to spend on their top 10 projects, with the total spent on each project once all 28 participants had voted yielding an overall "Top 10" list representing what people living with sickle cell disease would like the research community to focus on.

SCD TOP TEN RESEARCH TOPICS

Lived experience and the SCD research agenda

METHODOLOGY FOR A PRIORITY SETTING WORKSHOP WITH SCD PATIENTS AND CARERS, ASCAT 2019

Step **One**: introductions

Step **Two**: Presentation: What is PPI and why does it matter?

Step **Three**: Have a think. What questions would you like research to answer? What problems do you have that you would like research to fix? Then write your thoughts on a post-it note

Step **Four**: put your post it notes on "the wall"

Step **Five**: we organise the ideas into 'themes'

Step **Six**: we discuss and agree research topics for each theme

Step **Seven**: You are given "100 million dollars" (in 10 post-it notes!)...

Step **Eight**: You have to decide which research topics will get your 100 million

Step **Nine**: We tally up and reveal the Top 10

Step **Ten**: Prepare and present your work to the conference delegates

And finally: Celebrate! And group debrief.

Devised and conducted by Oxford Blood Group, in association with EuroBloodNet

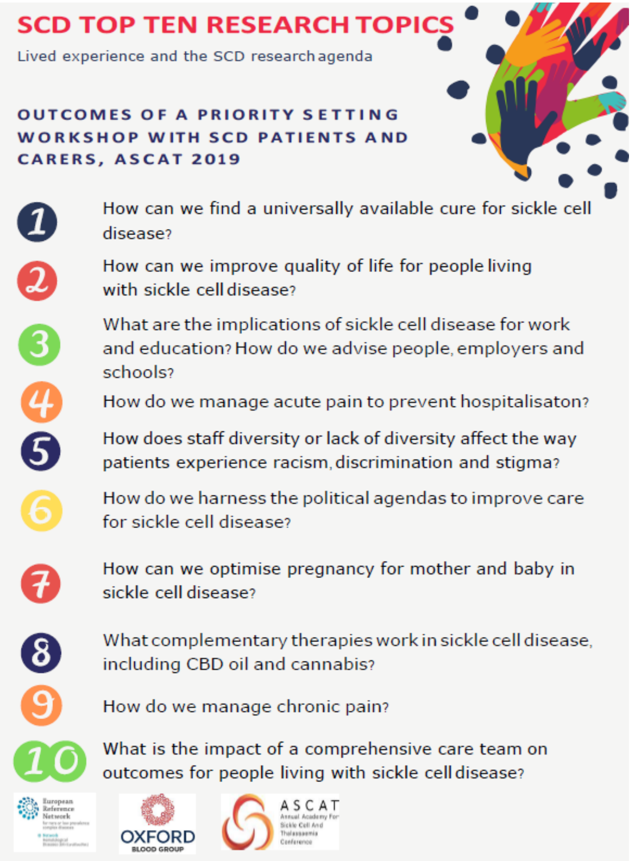
Fig. 5 Workshop's methodology in a glance

Results

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

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

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



SCD TOP TEN RESEARCH TOPICS

Lived experience and the SCD research agenda

OUTCOMES OF A PRIORITY SETTING WORKSHOP WITH SCD PATIENTS AND CARERS, 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 hospitalisaton?
5. How does staff diversity or lack of diversity affect the way patients experience racism, discrimination and stigma?
6. How do we harness the political agendas to improve care for sickle cell disease?
7. How can we optimise pregnancy for mother and baby in sickle cell disease?
8. What complementary therapies work in sickle cell disease, including CBD oil and cannabis?
9. How do we manage chronic pain?
10. What is the impact of a comprehensive care team on outcomes for people living with sickle cell disease?

Fig. 6 ASCAT 2019's workshop results

3. At the end of the workshop, a satisfaction questionnaire was submitted to the patients and the result of this questionnaire indicates a positive experience in unison. Questionnaire results in the Annex III_ASCAT 2019

4. Another important result to be underlined is the last year's session patients' testimonies. Those are collected in the following video published on ERN-EuroBloodNet Educational YouTube Channel: https://www.youtube.com/watch?v=RyjCL311DYw launched in occasion of the International Sickle Cell Disease International Day, the 19th of June 2020. (…) WSCD 2020 EuroBloodNet - SCD Research Prioritization Workshop at ASCAT Video: 5 patients from: Portugual, USA, Italy, Netherland, Cyprus have given testimony on the video.

- Video Is available: https://youtu.be/RyjCL311DYw

Here follows some rates obtained from the YouTube EuroBloodNet EDU Channel:

Video Title	Views	Likes	Number of displays	Watch time hours
WSCD 2020 EuroBloodNet - SCD Research Prioritization Workshop at ASCAT	194	3	304	7

Fig 7 In the table are reported the Interaction registered on the YouTube EuroBloodNet EDU Channel.

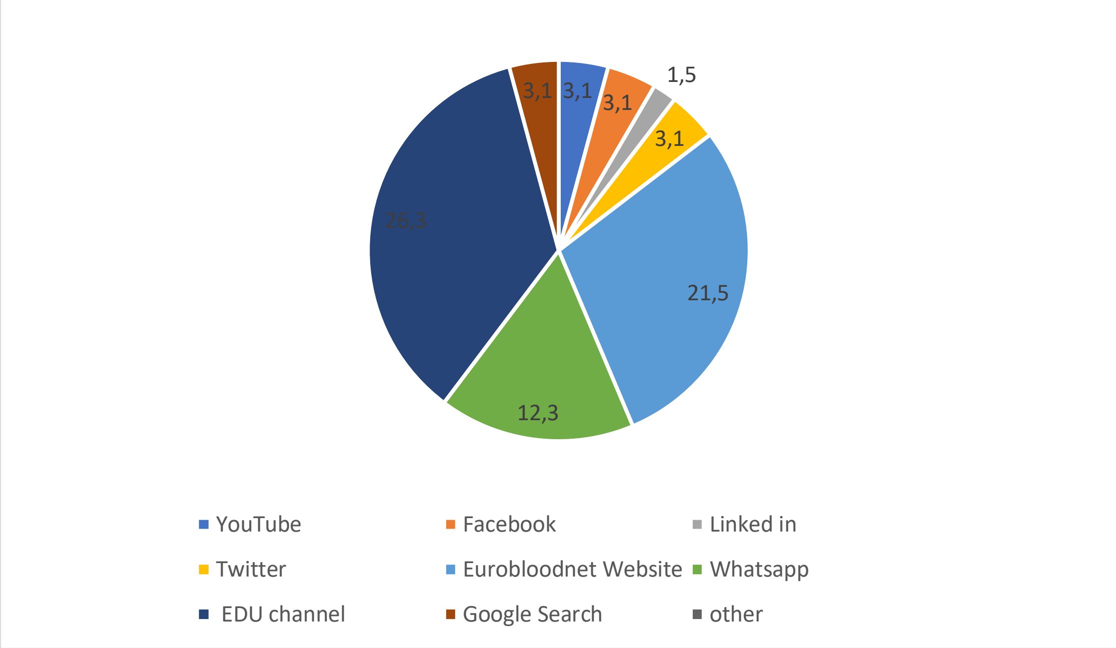


Fig. 8 Source of viewers traffic (%) of the video WSCD 2020 EuroBloodNet - SCD Research Prioritization Workshop at ASCAT on the YouTube EuroBloodNet EDU Channel.

5. Last result to report is the presentation of the poster "Pragmatic Approach to empowering patients with SCD: Top 10 Research Priorities from the European EuroBloodNet/ASCAT Network of SCD Patients" presented at the 25th Virtual European Hematological Association Congress (EHA), on June 11th - 21st, 2020. The poster presented at EHA is in "Annex IV_ASCAT EHA ".

SICKLE CELL DISEASE PATIENTS EDUCATIONAL SESSION AT ASCAT 2020

Objective

Considering the success of the SCD patients' session at ASCAT 2019, it has been 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 . This year the ASCAT Conference has been organized in collaboration with the European Heamatology Association (EHA) and the British Society of Haematology (BSH), from the 26th to 31st of October, on an online virtual platform as a results 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 will also give the possibility to assist to the whole ASCAT Congress event. This is 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 is dedicated this year to: ‘Haemoglobinopathies: Emerging Challenges and Future Therapies'.

Main idea is to create a SCD patients forum with about 100 patients coming from European countries and in a smaller part, SCD patients coming from extra communitarian countries. The creation of such a group (moreover if a part of this group had already the opportunity to get in touch thank to ASCAT past event) is capital for patients empowerment. It means more specifically: sharing opinion on burdens regarding living with SCD, sharing best practices and advocating at European and global level.

Methods

Together with ASCAT team, EuroBloodNet Association is planning for SCD patients an online Educative Session, programmed on three different days. A team of SCD experts belonging to ERN-EuroBloodNet and ASCAT committee is leading this educational session, whose coordination is still ongoing. 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 has 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.
- Living with SCD as pediatric or young adult patients.

As visible in the "Annex V_ASCAT 2020 Draft program", presenting the draft program of the ASCAT 2020 patients’ session, a duo made by physician and patient representative will moderate each topic session. Their role will be 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 will be shared. For better shaping the framework of each specific session, the ERN-EuroBloodNet Coordination Team is doing the exercise of collecting questions and key messages before the official day of the Educational SCD session at ASCAT.

The Educational Online Session will be fully recorded. Subtitles in different languages will be edited to the video. Those videos will be available on the YouTube Educational channel, EuroBloodNet Edu's:
https://www.youtube.com/channel/UC7011seQKL7STmLpWPmUFig

Expected Results

1. Editing the recorded SCD Educational Session with subtitles in different languages and make them available on the EuroBloodNet YouTube Channel.
2. Feedback Survey to the SCD Educational Session participants in order to understand how to improve the program in upcoming year.

At the actual stage of the action (The session is foreseen between the 26th-31st of October), the draft of the program has been prepared: Annex V_ASCAT 2020 Draft program

Next steps

3. ERN-EuroBloodNet had planned two patients’ sessions to be held at the fourth Global Congress on Sickle Cell Disease at UNESCO in Paris, April 2020. This event is postponed because of the Covid19 pandemic. This congress would have brought together medical experts and researchers experienced in Sickle Cell Disease from around the world. During the congress, two sessions of 2 hours each would have been dedicated to patient testimony. There would have been four presentations of patient testimony of 20 minutes each + 10 minutes of audience questions. This event would be reorganized.

4.1.2. SICKLE CELL DISEASE REPOSITORY OF EDUCATIONAL MATERIAL FOR PATIENTS

Rationale

The ERN-EuroBloodNet is currently addressing different Sickle Cell Disease patients’ needs across European Member through dedicated actions. As stated in the deliverable: “Deliverable 5.1 Report of educational gaps” of 2018, the collection of the educational material for SCD patients available within Network’s HCPs and among patients organizations across EU MS was identified as an urgent need. The analysis of the gap was undertaken by the ERN-EuroBloodNet coordination team, Angelo Loris Brunetta (ePAG RBC subnetworks coordinator), Dr Raffaella Colombatti (expert member of SCD), TFA coordinators of CME, and after gathering the opinion about patients ’needs among SCD Patients Association.

Objective

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

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

Methods

The SCD educational material for patients has been compiled from:

a) EuroBloodNet healthcare professionals: from Part 1 of the ERN-EuroBloodNet Questionnaire on continuing medical education (CME) as described in the deliverable : “Deliverable 5.1 Report of educational gaps” of 2018
b) SCD patients’ organizations across EU:

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

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

A questionnaire on the available educational material for SCD patients was already established, and designed by ERN-EuroBloodNet coordination team and Angelo Loris Brunetta (ePAG RBC subnetwork coordinator). It was prepared in an excel including two tasks:

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

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

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

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

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

The educational needs are expressed and classified by the following categories:

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

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

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

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

e) Other

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

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

Next step

- The survey will be simplified in order to obtain more answers
- The survey will be adapted in order to be harmonized with the Repository model for Patients educational Material.

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- SCD Videos produced in the framework of the Educational YouTube Channel “ERN-EuroBloodNet EDU”, like for instance the ASCAT 2020 Educational recorded sessions, will be integrated in the repository and consequentially in the e-learning platform.
- It could be translated in French, Spanish and Italian in order to reach a good number of patients’ organizations. Sickle Cell Diseases patients in Europe speak mostly English and French.
- The survey will be conducted during 2020. A report will be prepared with main results.
- A repository with sharable material will be created and made available on the EuroBloodNet e-learning platform.
- Eventual Therapeutic patient education needs expressed both by experts and by patients organizations will be addressed in the coming annual work plans in order to consider how to promote these needs and close the identified gaps.

4.1.3. SICKLE CELL DISEASE PATIENTS EDUCATIONAL VIDEO

Rational

The Spanish SCD patients association (Asociación Española de Enfermedad Falciforme, ASAFE) has proposed to the ERN-EuroBloodNet to develop Educational Video that would last between 5 and 10 minutes and whose speech would have been exposed by a SCD expert member of the Network. ASAFE had identified among its members 11 SCD related topics they considered needed to be addressed by visual pedagogical content.

The 11 identified topics were:

1. New therapies for SCD
2. Adult Patients Quality of Life
3. BMT: survival infertility and othercomplications
4. Neurogical Damages in SCD
5. Neonatal Screening
6. Gestational Risk
7. SCD and immune disease
8. Genetic Couselling…
9. Hydroxurea and fertility
10. Polynuria and Enuresis: kidney damages
11. Priapism

Objective

To keep the educational action at European level (and not simply targeted at Spanish level), the ERN-EuroBloodNet has launched a survey among all the European SCD Patients Associations in its contacts, in order to prioritize the 11 pre-identified topics and to give the possibility to other associations to propose new topics too. The main goal was to produce educational video that consider educational patients needs at European level.

Method

1. Prioritize the 11 SCD topics via a survey (ERN-EuroBloodNet's Survey for prioritizing SCD topics to be adressed by Educational Videos) to be conducted among SCD patients associations reached by the ERN-EuroBloodNet by previous actions.

2. Survey has been conducted by ERN-EuroBloodNet Coordination Team via EU Survey platform. Survey was available at the following link: https://ec.europa.eu/eusurvey/runner/EuroBloodNetSurveySCDvideoTOPICS
3. Each topic could be rated by a score from 1= not very important to 5= very important
4. ERN-EuroBloodNet SCD experts will perform the video in their native languages. Then subtitles in other European languages will be edited in order to reach the SCD patients community at large.

Results

“ERN-EuroBloodNet's Survey for prioritizing SCD topics to be adressed by Educational Videos” has gathered 24 answers expressed by SCD from: France, Italy, Spain, Portugal, Cyprus, USA, Belgium.

AVERAGE SCORE		
1	New therapies for SCD	4,56
2	Adult Patients Quality of Life	4,5
3	BMT: survival infertility and othercomplications	4,045
4	Neurogical Damages in SCD	4
5	Neonatal Screening	3,85
6	Gestational Risk	3,6
7	SCD and immune disease	3,56
8	Genetic Couselling…	3,52
9	Hydroxurea and fertility	3,47
10	Polynuria and Enuresis: kidney damages	3,4
11	Priapism	3,1

Fig. 9 Results from the Survey, by order of prioritization and average score.

Next steps

Video will be recorded in expert native language and subtitled with several European languages. Some topics could be eventually adressed by interactive webinars.

4.2 CUTANEOUS LYMPHOMA DEDICATED ACTIONS

4.2.1 COMPREHENSIVE WEBINAR PROGRAM ON CUTANEOUS LYMPHOMA FOR PATIENTS ASSOCIATIONS

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 TFA coordinators of CME, ePAGs and Eurordis has planned the strategy for tailoring the Webinar programs for patients. Strategy will be devoted for the definition of comprehensive programs compound on serveral sessions on very rare diseases given the higher necessity for in-depth knowledge sharing. First disease identified is Cutaneous Lymphoma (CL), where a task force of health professionals and patients advocates (including Coalition of patients organizations) have been identified willing to establish a Comprehensive program on CL for patients' organizations (see section 2.1.3), in collaboration with EURORDIS, and Lymphoma Coalition Europe and Cutaneous Lymphoma Foundation.

Objective

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

Methods

Webinars are online educational activities conducted using the Webex Platform provided to ERN-EuroBloodNet by the European Commission. It allows full audio and visual communication, as well as interaction between webinar's speaker and participants. Webinars are led by the speaker with the visual aid of a Power Point presentation and recorded by the coordination team. The mp4 audio got by the session and the power point presentation will be uploaded and made available on the ERN-EuroBloodNet webpage. At the end of the Patients webinar program a Power Point Presentation slide will mention the upcoming events organised by Patients associations, Networks, etc. with respect to the topic of the session.
According to the directive of the GDPR, speakers are requested to sign a presentation publishing agreement to authorize the publication of the webinar recorded together with the slides presentation on the ERN-EuroBloodNet website. Also, webinars 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.

Webinar target

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

Webinars speakers

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

Results:

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

Lymphoma Coalition Europe will share with us the result allowing us to build a truly tailored program on the real, practical and concrete needs of the patient. The ERN-EuroBloodNet Webinar comprehensive program on Cutaneous Lymphoma for patients' organisations will be tailored and based on the 2020 Lymphoma Coalition Global Patient Survey on Lymphomas and CLL for guarantee that needs expressed by patients and caregivers in the survey will be listened and tackled.

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

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

Disease oriented

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

General

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

Co-funded by the European Union

    c) Systemic treatments (how can I prepare what can I expect?)*
        a. Oral*
        b. Infusion*
6. Everyday:
    a) Nutrition
    b) Sexuality
    c) Other tips and tricks

* If applicable

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

Next steps

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

4.2.2. CUTANEOUS LYMPHOMA REPOSITORY OF EDUCATIONAL MATERIAL FOR PATIENTS

Rationale

As stated in previous introduction, the ERN-EuroBloodNet is creating an e-learning platform that will incorporate educational material dedicated to some specific Rare Hematological Disorders and targeted for health professionals and patients. Within this framework it is foreseen the creation of the repository of Cutaneous Lymphoma (CL) educational material for patients.

Objectives

The repository material has a double utility:

- A landscape analysis for understanding which pedagogical areas of CL have already been covered and identifying the gaps. The Coutaneous Lymphoma Webinar comprehensive program, described in the previous paragraph could address eventual identified gaps.
- Make the educational material accessible for patients through the repository itself.

Methods

ERN-EuroBloodNet Coordination Team, CL or Lymphoma patients associations (Europe Lymphoma Coalition, France Lymphoma Espoir, Cutaneous Lymphoma Foundation) and EURORDIS have conducted a first analysis of the existing available educational material for CL. A first list of the educational material was collected and classified on an Excel file and shared with the CL task force for a second revision. This list was created by collecting material from: Cutaneous Lymphoma Foundation, Lymphoma Coalition Europe, France Lymphoma Espoir.

Material has been distinguished between:

- CL subtype/Generic CL
- Title of the educational material
- Topic Category: (quality of life/treatment/Diagnosis, etc)
- Adult/Pediatric/Young adult/all
- Type of document (PDF, PPT, Leaflet, Book, etc)
- Language/s of the material available L
- ink or indicate annex (PDF, PPT)
- Contact the organisation (if material is not on the repository)

All the material collected were documents approved by CL experts and CL patients advocates.

Results

Collected educational material has been gathered in a Excel.

Next steps

- Organizing a second revision of the repository adding the CL educational Material for patient produced by Lymphoma Action in the UK and Lymphoma Switzerland.
- Adding the video recorded from the Comprehensive Webinar program
- Integrating the Excel file on the ERN-EuroBloodNet website through the e-learning platform

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. Establish a Comprehensive Webinar program for patients on HH-IRON.
2. Producing Educational Visual Material based on a) research results / b) treatments options & Guidelines --- return to patient.
3. Creating Patients summery based on Clinical Practice Guidelines.
4. Establishing Patients Journey. It has been proposed the possibility to establish a patient journey on Von Willebrand Disease.
5. Coordination of patients educational and advocacy trainings.
6. Coordination of patients' sessions at congresses.

6 . CONCLUSIONS

One of the most ambitious objective of the ERN-EuroBloodNet is the promotion of patients' empowerment targeting both patients and patients associations, in the field of the RHDs. For this aim is essential the collaboration with EURORDIS, European Patients Networks, Patients organizations at National and local level, not only for identifying needs and plan potential actions, but for its analysis and development, in order to achieve more efficient results.

The projects undertaken by the network during the third period of the ERN have allowed tackling gaps previously identified by the establishment of patients empowering programs that crossed the axes of Patients Advocacy, Cross Border Health Assistance and Patients Education.

1. Patients Advocacy
   a. The Mapping of Patients organizations across Europe and the identification of ePAGs advocates, in order to promote patients' rights, access to information and healthcare services at European Level.
   b. The national meetings organized for the establishment of the European Network of Sickle Cell Disease Patients organizations that allow the introduction of patients in the "advocacy" meaning of the word while initiating them into the main steps to be part of the ePAG and ERNs community.
2. Cross Border Health Rights
   a. 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.
   b. A preliminary analysis of Cross Border Health access to blood transfusions through surveys.
3. Patients Education
   SCD related actions

   a. Sickle Cell Disease Research Prioritisation Workshop at ASCAT 2019 that trained patients to understand their role on research. Moreover, the organization of Sickle Cell Disease Research Prioritisation Workshop at ASCAT, provided with the top 10 priorities to be most urgently tackled, allowing the identification of direct and indirect educational actions as: What are the implications of sickle cell disease for work and education? How do we advise people, employers and schools? How do we manage chronic pain?
   b. Sickle Cell Disease Patients Educational Session at ASCAT 2020: 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.
   c. Sickle Cell Disease Repository of Educational Material for patients that will create an accessible archive of multilingual document available on SCD related educational topics.
   d. Sickle Cell Disease Patients Educational Video that will increase SCD patients awareness by creating videos

   Cutaneous Lymphoma related actions

e. Definition of Webinars program on Cutaneous Lymphoma for patients’ advocates and patients association in order to understand diagnosis, treatments and management of specific type of Cutaneous Lymphoma to be better prepared to support within his/her patients’ organization newly and already diagnosed patients.
f. Cutaneous Lymphoma Repository of Educational Material for patients that will establish an accessible list of multilingual educational document on Cutaneous Lymphoma related educational topics.

In conclusion, ERN-EuroBloodNet Patients empowerment strategy has allowed not only to directly promoting patient-centred projects. Nevertheless, it has produced different educational programs addressed to patient community at large and patients associations. ERN's Patients' Plan has also initiated SCD patients in the advocacy environment, and increased the European Patients Group Representation within the Network and offered Cross Border Health Assistance. Considering those fruitful results and outcomes obtained from the initiatives, same actions coverage will be applied to other RHD areas already identified as urgent targets to be tackled, as for instance the possibility of establishing an European Network of Cutaneous Lymphoma Patients Associations, or the expansion of the comprehensive webinars program to Haemochromatosis or other iron disorders. In addition new actions has been proposed for integrate an exhaustive strategy of patients’ empowerment, i.e. for quoting one of the most ambitious, the production of patients journeys.

ANNEX I

OUTCOMES FROM NATIONAL MEETINGS

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Hematological
Diseases (ERN EuroBloodNet)

Establishment of a European Network of Sickle Cell Disease Patients Organisations across EU MS

Outcomes

1. **First national meeting in Milan, the 29th of March 2019**
2. **Second National Meeting, in Paris, the 29th of June 2019**
3. **Third National Meeting, in Madrid, gathering Spanish and Portuguese patients, 18th February 2020**

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

EURORDIS
RARE DISEASES EUROPE

Establishment of a European Network of Sickle Cell Disease Patients Associations across EU MS
Meeting with Italian PO’s
29th of March 2019

Venue: Aula Polo Scientifico, Fondazione Ca’ Granda Ospedale Maggiore Policlinico (IRCCS), Via Francesco Sforza, 35 , 20122 Milano

AGENDA

- **10.00-10.30** Welcome coffee
- **10.30-10.45** Tour de table of the participants
- **10.45-11.00** Introduction of ERN-EuroBloodNet (Mariangela Pellegrini)
- **11.00-11.30** ePAGs role in RDs panorama (Loris Brunetta)
- **11.30-11.45** Physicians engagement for the ERN EuroBloodN (Raffaella Colombatti)

- **11.45-12.00** Coffee break

- **12.00-12.30** Characteristic of the European Network of SCD patients association
    - Network description (Mariangela Pellegrini)
    - Objectives and added values of having an European Network (Loris Brunetta)
    - Network's patient representatives "should have" criteria (Loris Brunetta)
    - Training with… (Eupati, Eurordis, EuroBloodNet ePAGs) (Loris Brunetta)

- **12.30-13.30** Lunch break

- **13.30-14.45** PO’s presentation: organizations and actions for SCD, *15 min*

  - United Onlus

  - Associazione Fondazione Italiana ‘L.Giambrone' for Thalassemia

  - Associazione Malattia Drepanocitica

  - EXPO IME EKEMINI

  - Associazione Italiana Drepanocitici di Torino

- **14.45-16.00** PO’s feedbacks and questions

- **16.00-16.30** Contacts of those PO’s which are interested being engaged in the project

  →**Next steps**: Italian patients associations communicate who is their national representative and substitute.

Discorso introduttivo

- **Presentazione dell'ERN-EuroBloodNet**: come nasce l'iniziativa, obiettivi e progetti, la focalizzazione sulla drepanocitosi.
  Presentazione ppt in allegato.

- **Presentazione del ruolo dei pazienti all'interno dell'ERN**: il compito degli ePAG, qual é il valore aggiunto di un'iniziativa centrata sul punto di vista del paziente, la formazione degli ePAG, la voce della comunità europea dei pazienti rappresentata dagli ePAG
  Presentazione ppt in allegato

- **Presentazione del ruolo dei medici all'interno dell'ERN EuroBloodNet**: la collaborazione per lo stabilimento del registro di anemie rare, l'utilizzo della piattaforma di telemedicina CPMS, l'analisi della direttiva sulle cure transfrontaliere (il caso del trapianto di midollo osseo tra Italia e Irlanda per pazienti drepanocitici di fascia pediatrica), il raggruppamento di linee guida e buone pratiche, l'educazione medica continua e l'educazione terapeutica del paziente. *Presentazione ppt in allegato.*

- **Sito Web EuroBloodNet**: www.eurobloodnet.eu   Siamo presenti anche su twitter

Presentazione del progetto: Rete europea di associazioni di pazienti drepanocitici

- **Come sarà la rete europea di associazioni di pazienti drepanocitici?**
  - La rete unirà le associazioni nazionali, regionali, locali che vorranno far parte della rete europea.
  - EuroBloodNet darà l'endorsement EuroBloodNet alla rete, ma qualora la rete voglia costituirsi in un' alleanza o una federazione, e avere quindi uno statuto legale di associazione sarà libera di farlo, autonomamente.

- **Che cosa chiede EuroBloodNet alle associazioni nazionali dei vari paesi?**
  - *Obbligatorio*. Le modalità per identificare i due rappresentanti saranno scelte dalle associazioni italiane. EuroBloodNet raccomanda che siano persone motivate, capaci di parlare in inglese e che conoscano la realtà sociale, giuridica e clinica in Italia, per i pazienti drepanocitici. Perché questo avvenga è in atto un percorso di formazione condiviso.

  - *Facoltativo.* Alle associazioni nazionali, regionali o locali di un dato paese europeo EuroBloodNet suggerisce che vengano federate, formalmente o no, a livello nazionale per facilitare il flusso delle comunicazioni e la concentrazione delle risorse e delle conoscenze. Questo passaggio è facoltativo.

  - *Obbligatorio*. I rappresentanti nazionali di tutte le associazioni europee (il comitato della rete europea) dovranno scegliere chi sarà il rappresentante europeo dell'associazione, che diverrà inoltre ePAG per EuroBloodNet. Le modalità di selezione saranno liberamente scelte dal comitato della rete europea delle associazioni di pazienti affetti da drepanocitosi. Ciò avverrà entro il termine del percorso di formazione in tutti i paesi europei (tre anni).

- **Come contribuirà EuroBloodNet alla rete europea?**
    - EuroBloodNet si occupa della creazione della rete europea delle associazioni di pazienti drepanocitici. Organizza i primi meeting nazionali, mette in contatto le realtà nazionali e raggruppa i rappresentanti nazionali nel primo meeting europeo. Queste azioni sono coperte finanziariamente da EuroBloonet. Una volta che la rete sarà lanciata, dovrà essere autonoma.
    - EuroBloodNet offre lo spazio alla rete europea delle associazioni di pazienti drepanociti sulla sua pagina web
    - EuroBloodNet offre visibilità sui suoi social media
    - EuroBloodNet offre dei seminari formativi con gli ePAGs di EuroBloodNet e altre organizzazioni internazionali e nazionali, che saranno in grado di spiegare: come gestire un'associazione europea dalla comunicazione alla ricerca dei finanziamenti, come formarsi al ruolo di advocate, come interagire con le ERN.
    - EuroBloodNet offre l'informazione sul panorama europeao delle malattie rare.

- **Come contribuirà la rete europea di associazioni di pazienti alla causa della drepanocitosi?**
    - La rete europea delle organizzazioni di pazienti affetti da drepanocitosi potrebbe avere un ruolo chiave da svolgere nel settore sanitario europeo e di liason con la comunità medico-scientifica.
    - Aiuterebbe i pazienti e le persone che li assistono a comprendere i migliori percorsi dei pazienti e le buone pratiche.
    - Sarebbe un infopoint per diffondere informazioni su come e dove ottenere l'accesso alle migliori cure e promuovere la consapevolezza dei diritti dei pazienti e l'accesso alle cure sanitarie in quanto cittadini europei. Infine divulgherebbe conoscenza su come muoversi nel sistema sanitario per ottenere i migliori risultati possibili a livello nazionale ed europeo.
    - Fungerebbe da punto di contatto centralizzato a livello europeo per i pazienti, i familiari e i professionisti della sanità.
    - Infine, attuerebbe la relazione sulle politiche sulle malattie rare per garantire che tutte le politiche sanitarie e la ricerca rispondano alle esigenze e alle priorità dei pazienti.

- **Bisogni e aspettative espressi dai pazienti delle associazioni italiane durante la riunione**
    - È stata espressa vivamente la necessità di un maggior dialogo e confronto sulla malattia e sulle necessità dei pazienti, con i medici curanti e la comunità medico-scientifica del centro di cura, cosi come con quella nazionale ed internazionale. E' auspicata la definizione nel prossimo futuro di iniziative congiunte pazienti-medici/operatori sanitari, anche in collaborazione con le società scientifiche nazionali

- È stato condiviso l'isolamento che i pazienti sentono nei vari centri, nei quali spesso manca un ambulatorio dedicato per i pazienti drepanocitici che vengono visitati in day hospital o ambulatori insieme con pazienti oncologici o emato-oncologici, con cui non condividono bisogni e problematiche. Viene suggerita una maggiore specificità e viene richiesta una maggiore collaborazione da parte del personale sanitario per mettere in comunicazione i pazienti tra di loro e avvicinarli alle associazioni, li dove presenti.
- Combattere gli stereotipi legati allo stigma del paziente drepanocitico, in Italia e in Africa. Questo vuol dire lottare contro l'idea che la malattia sia una vergogna o una maledizione, informare sulle nuove aspettative di vita e il miglioramento della qualità di vita del paziente, informare sul fatto che non è semplicemente una patologia africana ma che è presente sul territorio mondiale. Sensibilizzare i familiari, insegnanti e datori di lavoro sulle implicazioni di questa patologia.
- Informare sui diritti in materia sanitaria del paziente drepanocitico. Come per esempio l'articolo sui permessi retribuiti: legge 104 articolo 3, comma 3.
- Promuovere la transizione dall'età pediatrica all'età adulta in ogni ambito: servizi clinici, strutture ospedaliere, materiale informativo per i pazienti, materiale informativo per i medici.
- Migliorare il percorso di presa in carico del paziente, soprattutto per l'urgenza/emergenza e l'accesso al Pronto Soccorso. Spesso il paziente con crisi dolorosa vaso-occlusiva viene fatto attendere per ore, non viene riconosciuto il codice giallo, non esiste percezione della gravità delle complicanze acute da parte del personale sanitario. Vengono proposte alcune idee: survey sull'accesso alle urgenze da condurre in collaborazione con le società scientifiche, una tessera di identificazione paziente da mostrare ai professionisti della sanità dove viene spiegata brevemente la presa in carico da eseguire, etc.
- Sensibilizzare all'accompagnamento psicologico.
- Promuovere azioni mirate in Africa tramite fondi europei.
- Creare nuove associazioni di pazienti a livello locale e regionale.

- **Prossimi passi**
    - A partire dai contatti inviati da Mariangela Pellegrini, ERN project manager, dei partecipanti alla riunione di Milano, creare una mailing list.
    - Comunicare a Mariangela Pellegrini, ERN manager, se ci sono altre associazioni italiani che andrebbero inserite nella mailing list.
    - Diffondere il verbale tra le associazioni nazionali, regionali, locali di pazienti drepanocitici al fine di informarli sulla creazione della rete europea. Inoltre il verbale sarà inviato alle società scientifiche italiane: SITE e AIEOP .
    - Organizzare una teleconferenza delle associazioni italiane, al fine di decidere come portare avanti il progetto. Mariangela Pellegrini, ERN project manager, si occuperà della organizzazione della teleconferenza.
    - Creare una eventuale federazione informale o formale italiana.
    - *Facoltativo* Partecipazione agli eventi dedicati alla drepanocitosi in quando gruppo delle associazioni di pazienti italiani (per esempio il 19 giugno, giornata internazionale della drepanocitosi).

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

EURORDIS
RARE DISEASES EUROPE

Création d'un réseau européen d'associations de patients atteints de drépanocytose dans les États membres de l'UE

Rencontre avec les organisations françaises de patients
29 juin 2019

Lieu

Hôpital Saint-Louis, 1, Avenue Claude Vellefaux, Paris 75010.
Salle de réunion Trèfle 3, PLOT B, troisième ètage.

ORDRE DU JOUR

- 9.00-9.30 Café de bienvenue
- 9.30-9.45 Tour de table des participants
- 9.45-10.10 Introduction d'ERN-EuroBloodNet (Mariangela Pellegrini, ERN EuroBloodNet chef de projet)
- 10.10-10.30 Caractéristiques du Réseau européen des associations de patients SCD et les critères des représentants des patients (Mariangela Pellegrini)

10.30-10.45 Pause-café

- 10.45- 11.15 Formation avec... (Eupati, Eurordis, EuroBloodNet ePAG) (Ariane Weinman, EURORDIS)
- 11.15-11.45 Rôle des ePAGs dans le panorama des MR (Jean-Philippe Plançon, ePAG dans l'ERN EURO-NMD)
- 11.45- 12.15 Présentation de comment la filière représente les associations au niveau national (Sonia Pavan, Chef de projet Filière de Santé MCGRE)

12.15-13.15 Pause déjeuner

- 13.15-16.00 Présentation des associations présentes : organisations et actions, 10 min
    - *Présentations des associations*
- 16.00-16.30 Commentaires et questions des associations
- 16.30-17 Contacts des organisations intéressées par le projet

Discours d'introduction

- Présentation d'ERN-EuroBloodNet : Origine des ERNs, Structure et acteurs d’EuroBloodNet, objectifs et projets, accent sur les activités en faveur des patients atteints de drépanocytose.
  Présentation ppt en pièce jointe.

- Présentation du rôle des patients au sein de l'ERN : Missions de l'ePAG, formation de l'ePAG, témoignage de la communauté européenne des patients représentée par les ePAGs, valeur de la représentation du patient au niveau européen. Le porte-parole européen du Reseau européen des associations de patients atteints de drépanocytose pourra devenir ePAG pour l’ERN-EuroBloodNet.
  Présentation ppt en pièce jointe

- Présentation d’EURORDIS : Naissance et développement d'Eurordis au fil des ans. Rôle, missions et actions menées par Eurordis. Formations possibles offertes par Eurordis et EUPATI pour devenir un patient expert et agir dans le paysage européen. Eurordis participe avec EuroBloodNet à la création d’un réseau européen de patients atteints de drepanocytose.
  Présentation ppt en pièce jointe

- Présentation de la filière MCGRE : Organisation de la filière, missions, présentation des groupes de travails, relations et actions avec les associations des patients, nouveau site web. Le réseau a coordonné la réunion des associations françaises avec EuroBloodNet. La filière MCGRE veillera à l’harmonisation des actions des associations de patients au niveau français et européen. La filière accompagnera les actions des associations françaises au sein du réseau européen et veillera à ce que les communications du réseau européen des associations de patients soient également réparties entre les associations françaises.
  Présentation ppt en pièce jointe

- Site Internet d'EuroBloodNet : www.eurobloodnet.eu
- Site Internet Eurordis https://www.eurordis.org/
- Site Internet ERN EURO-NMD https://ern-euro-nmd.eu/
- Site Internet filière MCGRE https://filiere-mcgre.fr/

Présentation du projet : Réseau européen d'associations de patients atteints de drépanocytose

A quoi ressemblera le réseau européen des associations de patients drépanocytaires? Le réseau rassemblera les associations nationales, régionales et locales qui souhaitent faire partie du réseau européen. EuroBloodNet donnera son aval au réseau de patients EuroBloodNet, mais si le réseau veut former une alliance ou une fédération et donc avoir un statut juridique d'association, il sera libre de le faire. Il est souhaitable que chaque pays ait un (ou deux) porte-parole et un (ou deux) suppléants en représentation de toutes leurs organisations nationales de patients. Parmi tous les porte-paroles, un représentant du réseau européen sera élu et deviendra ePAG pour le réseau ERN EuroBloodNet.

Comment le réseau européen des associations de patients contribuera-t-il à la cause de la drépanocytose ?

- Le réseau européen des associations de patients atteints de drépanocytose pourrait jouer un rôle clé dans le secteur européen de la santé et dans la liaison avec la communauté médicale et scientifique.
- Le réseau européen des associations de patients atteints de drépanocytose veillera que toutes les politiques européennes et la recherche en santé en Europe répondent aux besoins et aux priorités des patients.
- Le réseau européen des associations de patients atteints de drépanocytose sera un point de contact centralisé au niveau européen pour les patients, leurs familles et les professionnels de la santé.
- Le réseau européen des associations de patients atteints de drépanocytose pourra aider les patients et leurs soignants à comprendre les meilleures pratiques des patients et les meilleures pratiques des professionnels de la santé.
- Le réseau européen des associations de patients atteints de drépanocytose contribuera à améliorer l'accès aux meilleurs soins et à promouvoir la sensibilisation aux droits des patients en tant que citoyens européens.

Comment EuroBloodNet contribuera-t-il au réseau européen ?

- EuroBloodNet est responsable de la création du réseau européen des associations de patients drépanocytaires. EuroBloodNet organise les premières rencontres nationales, identifieles réalités nationales et réunit les représentants nationaux lors de la première rencontre européenne. Ces actions sont couvertes financièrement par EuroBloodNet. Une fois le réseau lancé, il devra être autonome.

- EuroBloodNet accompagnera les actions du réseau européen des associations de patients atteints de drépanocytose.

- EuroBloodNet offre l'espace au réseau européen des associations de patients drépanocytes sur sa page web.

- EuroBloodNet offre une visibilité sur ses médias sociaux.

- EuroBloodNet propose des séminaires de formation avec les ePAGs d'EuroBloodNet qui pourront expliquer : comment gérer une association européenne (communication, financement, organisation, etc.), comment interagir avec les ERNs, etc.

- EuroBloodNet offre l'expertise sur le paysage européen des maladies rares.

Besoins et attentes exprimés par les patients des associations françaises lors de la réunion

La nécessité d'un dialogue et d'une confrontation accrus sur la maladie et les besoins des patients a été fortement exprimée, tant avec les médecins traitants et la communauté médico-scientifique du centre de traitement, qu'avec la communauté nationale et internationale. Le rôle des associations de patients peut être le fondement pour garantir une médiation entre le patient et le médecin au niveau national et européen.

- Avoir une permanence des associations de patients atteints de drépanocytose au sein des hôpitaux.
- Plaidoyer au niveau européen.
- Former des patients experts.
- La création au niveau national d'une Fondation de Recherche dédiée à la drépanocytose.
- La généralisation du dépistage néonatal au niveau national.
- Sensibiliser à l'accompagnement psychologique.
- Sensibiliser à l'écoute du corps : par exemple aborder les crises avec des approches non pharmacologiques (massages, sophrologie, etc.).
- Lutter contre l'isolement des patients.
- Favoriser les rencontres entre patients drépanocytaires.
- Aider les migrants sans abris atteints de drépanocytose.
- Reconnaissance de la drépanocytose en tant que maladie lourde au niveau national et européen.
- Reconnaissance de la journée mondiale de la drépanocytose + création d'une journée européenne de la drépanocytose.
- Promouvoir des actions ciblées en Afrique.
- Lutter contre la stigmatisation du patient.
- Sensibiliser le milieu scolaire.

Les points suivants, basés sur les réunions tenues en France, en Italie et en Belgique, sont communs entre les pays et peuvent être traduits en actions concrètes au niveau européen

➔ PLAIDOYER AU NIVEAU EUROPEEN

1. Axe Politique de la Santé : reconnaissance de la drépanocytose en tant que pathologie lourde (le passage d'un handicap invisible à une pathologie lourde implique des effets dans tous les milieux sociaux : médical, scolaire, lieu de travail, etc.).
2. Axe sociétal : lutter contre la stigmatisation.
3. Axe social : lutter contre l'isolement des patients.
4. Axe bonnes pratiques :
   - 4.1 Utilisation de la carte d'urgence française (Filière MCGRE) au niveau européen (traduction dans les langues officielles de la EU).
   - 4.2 Sensibilisation à l'aide psychologique.
   - 4.3 Programmes de dépistage néonatal + prénatal.
5. Axe communication : Journée mondiale de la drépanocytose + Journée européenne.
6. Axe éducatif : Matériel d'information pour les patients et les professionnels de la santé. L'ERN-EuroBloodNet est en train de créer un répertoire de matériel éducatif, cette action peut être menée en commun.

Prochaines étapes

- A partir des contacts de la filière MCGRE (membres et non membres de la filière MCGRE) des associations des patients atteints de drépanocytose qui souhaitent participer au réseau européen, Mariangela Pellegrini, chef de projet ERN EuroBloodNet, va créer une liste de diffusion.
- Diffuser les conclusions auprès des associations nationales, régionales et locales de patients drépanocytaires afin de les informer de la création du réseau européen.
- Organiser les élections des 2 représentants et les 2 suppléants.

Elections des représentants nationaux

- Compte tenu de la fréquence de la drépanocytose en France, il a été choisi par l'ERN-EuroBloodNet, la filière MCGRE et Eurordis que la France ***ait 2 représentants et 2 suppléants nationaux.***

- Comme convenu lors de l'assemblée du 29 juin 2019, un délégué de l'Outre-mer et un délégué de la métropole devraient être représentants à égalité. Pour cette raison, seront élus : 1 représentant et 1 suppléant pour le territoire d'outre-mer et 1 représentant et 1 suppléant pour le territoire métropolitain.

Obligations des représentants et suppléants

1) Les représentants et suppléants s'engagent à représenter toutes les associations françaises. L'information devra être distribuée également à toutes les associations locales, régionales et nationales. Les représentants et suppléants nationaux doivent veiller à ce que l'ensemble de la communauté française soit représentée et entendue. Aucune association ne doit prendre le pas sur une autre ou empêcher la participation de certains membres.

2) Les personnes élues ne pourront, en aucun cas, se prévaloir de leur statut pour faire valoir des actions personnelles et/ou promouvoir l'action de l'association dont elles sont membres. Ce mandat a une visée de représentation strictement collective des associations de patients atteints de drépanocytose.

3) Les représentants et suppléants s'engagent à coopérer avec le réseau MCGRE, même si le représentant élu est membre d'une association qui n'est pas membre de la filière MCGRE. Si tel est le cas, l'association n'est pas obligée de devenir membre de la filière.

4) Les représentants devront participer aux réunions européennes et veiller à ce que la France soit représentée au sein du Comité européen (représentants nationaux d'autres pays).

5) Les représentants devront coordonner un plan stratégique national, en accord avec les associations françaises et en collaboration avec la filière MCGRE. Les choix stratégiques nationaux seront portés au niveau européen.

6) Les représentants français doivent coopérer avec les autres représentants nationaux et réaliser les missions et objectifs du réseau européen.

7) S'assurer qu'un représentant français est présent à un nombre minimum de réunions ou de téléconférences (80%).

8) Participer aux groupes de travail qui seront établis.

9) Participer à la rédaction des rapports annuels ou de planning annuels.

10) Participer activement à la vie du réseau européen des associations de patients atteints de drépanocytose.

11) Si les règles suivantes ne sont pas respectées, le rôle du représentant sera révoqué.

Qui peut se présenter comme candidat ?

1) Un patient ou parent d'un patient.
2) Une personne qui fait partie d'une association française dédiée à la drépanocytose. L'association peut être membre ou pas de la filière MCGRE.
3) Une personne motivée.
4) Capable de parler anglais.
5) Avec expérience de plaidoyer par exemple dans l'un de ces domaines : service sociale, santé publique, droit ou soins holistique, milieu clinique, accompagnement psychologique en France ou en Europe.
6) Intérêt pour participer aux formations pour les patients experts.
7) Le candidat ne doit pas avoir de conflits d'intérêt (ex: contrat avec l'industrie pharmaceutique)
8) Etre à l'aise avec la prise de parole en public
9) Intérêt pour le panorama européen de maladies rares

Comment les élections seront-elles gérées ?

1) La filière MCGRE demandera aux associations dont elle a connaissance (membres et non membres de la filière), si un de leurs adhérents souhaitent se présenter en tant que candidat.
2) Les candidats seront rendus publics par la filière MCGRE. Le candidat pourra présenter une lettre de motivation à sa participation (facultatif, mais encouragé parce que les

votants ne vont pas forcément connaître les candidats, et donc auront peut-être du mal pour faire leur choix).

3) Les élections seront gérées par la filière MCGRE via SurveyMonkey ou Google Form. Il y aura une élection pour le candidat de la France Outremer et une élection pour le candidat de la France métropole. Un vote pour associations. La date pour présenter sa candidature et pour voter vous sera communiquée à la rentrée.

Politique de référence pour le Conflit d'Intérêt

4) Les représentants nationaux doivent se conformer à la politique sur les conflits d'intérêts des ERNs.
5) Le candidat ne doit pas présenter de conflits d'intérêt, comme par exemple être employé par une société pharmaceutique.

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

EURORDIS
RARE DISEASES EUROPE

Establecimiento de una Red Europea de Asociaciones de pacientes con anemia falciforme

Meeting con los pacientes de Portugal y España
18 de Febrero 2020

Lugar:
Hospital materno infantil, planta 0, Aula 1
Hospital G. Universitario Gregorio Marañón. C/ Maiquez 5, 28007 Madrid.

AGENDA

- 10.00-10.30 Café de bienvenida
- 10.30-10.45 presentación de los participantes
- 10.45 a 11.00 Introducción de la ERN-EuroBloodNet
- 11.00 a 11.15 Panorama general de los proyectos dedicados a la Anemia falciforme
- 11.15-11.30 ¿quién son los ePAGs y Eurordis?

- 11.30-12.20 Pausa

- 12.20 a 13.00 Característica de la Red Europea de Asociación de Pacientes de Anemia Falciforme
    - Descripción de la red
    - Objetivos y valores añadidos de tener una Red Europea
    - Los criterios para ser representantes de los pacientes de la red
    - Cursos para el empowerement del paciente (Eupati, Eurordis, EuroBloodNet ePAGs)

- 13.00-14.00 Pausa Comida

- 14.00-14.45 Presentación de las organizaciones de pacientes

- 14.45-16.00 Necesidades y expectativas de los pacientes hacia la red europea

- 16.00-16.30 Comentarios y preguntas

Introducción

Las presentaciones power point están en adjunto del email

- **Presentación de ERN-EuroBloodNet:** Origen de las ERN, Estructura y actores de la ERN-EuroBloodNet, objetivos y proyectos, presentación de las actividades dedicadas a la anemia falciforme.

- **Presentación del rol de los pacientes dentro de la Red**: Misiones delos ePAGs, formación de los ePAGs, valor de la representación de los pacientes a nivel europeo. El portavoz europeo de la Red Europea de Asociaciones de Pacientes de Anemia Falciforme podrá convertirse en ePAG de la ERN-EuroBloodNet.

- **Presentación de EURORDIS**: Nacimiento y desarrollo de Eurordis a lo largo de los años. Rol, misiones y acciones llevadas a cabo por Eurordis. Posibles cursos de formación ofrecidos por Eurordis y EUPATI para convertirse en un paciente experto y actuar en el panorama europeo. Eurordis participa con EuroBloodNet en la creación de una red europea de pacientes que sufren de anemia falciforme.

Presentación del proyecto: Red Europea de Asociaciones de Pacientes de Anemia Falciforme

- **¿Cómo será la Red Europea de Asociaciones de Pacientes con Anemia Falciforme?**
  La red reunirá a las asociaciones nacionales, regionales y locales que deseen formar parte de la red europea. EuroBloodNet respaldará la red de pacientes de EuroBloodNet, pero si la red quiere formar una alianza o federación y, por lo tanto, tener un estatus legal de asociación, será libre de hacerlo. Es deseable que cada país tenga un (o dos) portavoz y un (o dos) suplente que represente a todas sus organizaciones nacionales de pacientes. De entre todos los portavoces, se elegirá un representante de la red europea que se convertirá en el ePAG de la ERN EuroBloodNet.

- **¿Cómo contribuirá la red europea de organizaciones de pacientes a la causa de la anemia falciforme?**
  - La Red Europea de Organizaciones de Pacientes con Anemia Falciforme podría desempeñar un papel clave en el sector sanitario europeo y en el enlace con la comunidad médica y científica.
  - La red europea de organizaciones de Pacientes con Anemia Falciforme vigilará que todas las políticas europeas y la investigación sanitaria en Europa respondan a las necesidades y a las prioridades de los pacientes.
  - La Red Europea de Asociaciones de Pacientes con Anemia Falciforme será un punto de contacto central a nivel europeo para los pacientes, sus familias y los profesionales de la salud.
  - La Red Europea de Organizaciones de Pacientes con Anemia Falciforme podrá ayudar a los pacientes y a sus cuidadores a comprender las mejores prácticas para los pacientes y las mejores prácticas para los profesionales de la salud.

    - La Red Europea de Asociaciones de Pacientes con Anemia Falciforme contribuirá a mejorar el acceso a la mejor atención y a promover la conciencia de los derechos de los pacientes como ciudadanos europeos.

- **¿Cómo contribuirá EuroBloodNet a la red europea?**
    - EuroBloodNet es responsable de la creación de la Red Europea de Asociaciones de Pacientes con Anemia Falciforme. EuroBloodNet organiza los primeros encuentros nacionales, identifica las realidades nacionales y reúne a los representantes nacionales en el primer encuentro europeo. Estas acciones están financieramente cubiertas por EuroBloodNet. Una vez que la red sea lanzada, debería ser autónoma.
    - EuroBloodNet acompañará las acciones de la red europea de asociaciones de pacientes con anemia falciforme.
    - EuroBloodNet ofrece un espacio a la red europea de asociaciones de pacientes de anemia falciforme en su página web.
    - EuroBloodNet ofrece visibilidad en sus medios sociales.
    - EuroBloodNet ofrece seminarios de formación con los ePAGs de EuroBloodNet que explicarán: cómo gestionar una asociación europea (comunicación, financiación, organización, etc.), cómo interactuar con las ERNs, etc.
    - EuroBloodNet ofrece de desarrollar experiencia en el panorama europeo de las enfermedades raras.

Las necesidades y expectativas expresadas por los representante de pacientes durante la reunión

- Se ha subrayado la importancia de tener una asociación nacional para que los pacientes tengan una referencia y una representación a nivel nacional.
    - **ES**: Se ha elegido de fundar una asociación nacional (y ha sido fundada, la ASAFE)
    - **PT:** Una asociación nacional existe ya desde 27 años pero falta de voluntarios para ser activa y operativa en el territorio. El presidente de la asociación pide nuevos miembros para que la asociación no termine.
    - Necesidad de desarrollar un plan de sostenibilidad de la asociación
    - Necesidad de desarrollar un plan de visibilidad de la asociación
    - Necesidad de desarrollar una colaboración entre representantes de pacientes, médicos, psicólogos y asistentes sociales
- La promoción de los exámenes de despistaje neonatal y prenatal a nivel nacional.
- Sensibilización hacia los temas de la enfermedad en los entornos escolares.
- Sensibilización hacia la importancia de un apoyo psicológico.
- Luchar contra el aislamiento de los pacientes.
- Reconocimiento de la anemia falciforme como una enfermedad discapacitante a nivel nacional y europeo.
- Luchar contra la estigmatización del paciente.
- Tener más informaciones accesibles para el paciente, y especificadamente:
    - Sexualidad, fertilidad y embarazo

    - Trasplante de medula ósea
    - Dolor crónico y concepto de cronicidad
    - Mapeo de la evolución de la enfermedad de la diagnosis a la edad adulta (Patient Journey).
- La posibilidad de poder viajar o quedarse en otro país teniendo la posibilidad de acceder a centros expertos en anemia falciforme (por ejemplo facilitar la posibilidad de tomar parte a programas europeo de formación como el Erasmus para los jóvenes pacientes).
- Tener centros equipado para trasplante de medula ósea que no sea centros dedicados a enfermedades oncológicas.
- Han sido propuestos un programa de webinar educativos para pacientes y para profesionales de la sanidad.

Los siguientes puntos, basados en las reuniones celebradas con los representantes de pacientes de España, Portugal, Francia, Italia y Bélgica, son comunes a todos los países y pueden traducirse en medidas concretas a nivel europeo

1. **Eje de política sanitaria**
   Reconocimiento de la anemia falciforme como patología discapacitante (el paso de una condición invisible a una patología discapacitante implica efectos en todos los entornos sociales: médico, escolar, laboral, etc.).

2. **Eje social**:
   a. Lucha contra la estigmatización.
   b. Lucha contra el aislamiento de los pacientes.
   c. Lucha para el reconocimiento de los “caregivers”.

3. **Buenas prácticas :**
   a. Sensibilización sobre la asistencia psicológica.
   b. Programas de exámenes neonatal + prenatal.

4. **Eje de comunicación**:
   Mayor visibilidad a la enfermedad (por ejemplo tomando parte a iniciativas para el día Mundial de la Anemia Falciforme)

5. **Eje educativo**:
   Tener más material informativo para pacientes y profesionales de la salud.(La ERN-EuroBloodNet está creando un directorio de material educativo, esta acción puede llevarse a cabo conjuntamente)

Próximos pasos

- Difundir las conclusiones a las asociaciones nacionales, regionales y locales de enfermos y/o otros pacientes con anemia falciforme afín de informarles sobre la creación de la red europea de asociaciones de pacientes con anemia falciforme.
- Elegir los representantes nacionales.

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EURORDIS
RARE DISEASES EUROPE

Criação de uma Rede Europeia de Associações de Doenças de Drepanocitose

Encontro com doentes de Portugal e Espanha
18 de fevreiro 2020

Lugar: Hospital materno infantil, planta 0, Aula 1
Hospital G. Universitario Gregorio Marañón. C/ Maiquez 5, 28007 Madrid.

ORDEM DO DIA

- 10.00-10.30 Café de boas-vindas
- 10.30-10.45 introdução dos participantes
- 10.45 - 11.00 Introdução da ERN-EuroBloodNet
- 11.00-11.15 Visão geral dos projectos de drepanocitose
- 11.15-11.30 Quem são as ePAGs e Eurordis?

- 11.30-12.20 Intervalo

- 12.20 - 13.00 Rede Europeia de Associações de Doentes com drepanocitose
    - Descrição da rede
    - Objectivos e valor acrescentado de dispor de uma rede europeia
    - Os critérios para ser um representante dos doentes na rede
    - Cursos de capacitação dos doentes (Eupati, Eurordis, EuroBloodNet ePAGs)

- 13.00-14.00 Pausa para almoço

- 14.00-14.45 Apresentação das associações de doentes

- 14.45-16.00 Necessidades e expectativas dos doentes em relação à rede europeia

- 16.00-16.30 Comentários e perguntas

Introdução

As apresentações em Power Point estão em anexo ao e-mail

- **Apresentação da ERN-EuroBloodNet**: Origem da ERN, Estrutura e actores da ERN-EuroBloodNet, objectivos e projectos, apresentação das actividades dedicadas à drepanocitose.

- **Apresentação do papel dos doentes no âmbito da Rede**: Missões da ePAG, formação da ePAG, valor da representação dos doentes a nível europeu. O porta-voz europeu da Rede Europeia de Associações de Doentes com drepanocitose pode tornar-se um ePAG da ERN-EuroBloodNet.

- **Apresentação da EURORDIS**: Nascimento e desenvolvimento da Eurordis ao longo dos años Papel, missões e acções realizadas pela Eurordis Possíveis cursos de formação oferecidos pela Eurordis e pela EUPATI para ser tornar um doente especializado e actuar na cena europeia. A Eurordis está envolvida com a EuroBloodNet na criação de uma rede europeia de doentes com drepanocitose

Apresentação do projecto: Rede Europeia de Associações de Doentes com Drepanocitose

- **Como será a Rede Europeia de Associações de Doentes com Drepanocitose?**
  A rede reunirá as associações nacionais, regionais e locais que desejem fazer parte da rede europeia. A EuroBloodNet irá apoiar a rede de doentes da EuroBloodNet, más se a rede quiser formar uma aliança ou federação e, portanto, ter um estatuto legal de associação, será livre de o fazer. É desejável que cada país tenha um (ou dois) porta-voz e um (ou dois) suplente para representar todas as suas organizações nacionais de doentes. De todos os porta-vozes, será escolhido um representante da rede europeia para se tornar o ePAG da ERN EuroBloodNet.

- **Como irá a rede europeia de organizações de doentes contribuir para a causa da drepanocitose?**
  - A Rede Europeia de Associações de Doentes de drepanocitose poderia desempenhar um papel fundamental no sector da saúde europeu e na ligação com a comunidade médica e científica.
  - A Rede Europeia de Associações de Doentes para a drepanocitose velará por que todas as políticas europeias e a investigação no domínio da saúde na Europa respondam às necessidades e prioridades dos doentes.
  - A Rede Europeia de Associações de Doentes con drepanocitose será um ponto de contacto central a nível europeu para os doentes, as suas famílias e os profissionais de saúde.
  - A Rede Europeia de Associações de Doentes com drepanocitose poderá ajudar os doentes e os seus prestadores de cuidados a compreender as melhores práticas para os doentes e as melhores práticas para os profissionais de saúde.

  - A Rede Europeia de Associações de Doentes com drepanocitose contribuirá para melhorar o acesso aos melhores cuidados e promover a consciência dos direitos dos doentes enquanto cidadãos europeus.

- **Como irá a EuroBloodNet contribuir para a rede europeia?**
  - A EuroBloodNet é responsável pela criação da Rede Europeia de Associações de Doentes com drepanocitose. A EuroBloodNet organiza as primeiras reuniões nacionais, identifica as realidades nacionais e reúne os representantes nacionais no primeiro encontro europeu. Estas acções são financeiramente cobertas pela EuroBloodNet. Uma vez lançada a rede, esta deverá ser autónoma.
  - A EuroBloodNet acompanhará as acções da rede europeia de associações de doentes com drepanocitose.
  - EuroBloodNet oferece um espaço para a rede europeia de associações de drepanocitose no seu sítio Web.
  - A EuroBloodNet oferece visibilidade nas suas redes sociais.
  - EuroBloodNet oferece seminários de formação com a EuroBloodNet ePAGs que explicarão: como gerir uma associação europeia (comunicação, financiamento, organização, etc.), como interagir com as ARN, etc.
  - A EuroBloodNet oferece-se para desenvolver conhecimentos especializados no panorama europeu das doenças raras.

As necessidades e expectativas expressas pelos representantes dos doentes durante a reunião

- Foi sublinhada a importância de ter uma associação nacional para que os doentes tenham uma referência e uma representação a nível nacional.
  - ES: Foi escolhida uma associação nacional para ser fundada (e foi fundada a ASAFE)
  - PT: Há 27 años que existe uma associação nacional, mas faltam voluntários para serem activos e operacionais no território. O presidente da associação pede novos membros para que a associação não cesse.
  - Necessidade de desenvolver um plano de sustentabilidade para a associação
  - Necessidade de desenvolver um plano de visibilidade da associação
  - Necessidade de desenvolver a colaboração entre representantes dos doentes, médicos, psicólogos e assistentes sociais
- A promoção do rastreio neonatal e pré-natal a nível nacional.
- Sensibilização para as questões da doença em contextos escolares.
- Sensibilização para a importância do apoio psicológico
- Luta contra o isolamento do paciente.
- Reconhecimento da drepanocitose como uma doença incapacitante a nível nacional e europeu
- Luta contra a estigmatização do doente

- Para ter mais informação acessível ao paciente, e específicamente
    - Sexualidade, fertilidade e gravidez
    - Transplante de medula óssea
    - Dor crónica e conceito de crónica
    - Mapear a progressão da doença desde o diagnóstico até à idade adulta (Patient Journey).
- A possibilidade de poder viajar ou permanecer noutro país, tendo acesso a centros especializados em drepanocitose (por exemplo, facilitando a possibilidade de participar em programas de formação europeus como o Erasmus para jovens doentes)
- Ter centros equipados para transplante de medula óssea que não sejam centros dedicados a doenças oncológicas.
- Foi proposto um programa de webinars educativos para doentes e profissionais de saúde.

Os seguintes pontos, baseados em reuniões com representantes dos doentes de Espanha, Portugal, França, Itália e Bélgica, são comuns a todos os países e podem ser traduzidos em medidas concretas a nível europeu

1. Eixo da política de saúde
    - Reconhecimento da drepanocitose como patologia incapacitante (a transição de uma condição invisível para uma patologia incapacitante implica efeitos em todos os ambientes sociais: médico, escolar, laboral, etc.).

2. Eixo social:
    - Combater a estigmatização.
    - Lutar contra o isolamento do paciente.
    - Lutar pelo reconhecimento dos prestadores de cuidados.

3. Boas práticas :
    - Sensibilização para a assistência psicológica.
    - Programas de rastreio neonatais + pré-natais.

4. Eixo de comunicação:
    - Aumento da visibilidade da doença (por exemplo, participando em iniciativas para o Dia Mundial da drepanocitose)

5. Eixo educativo:
    - Dispor de mais material informativo para doentes e profissionais de saúde (a ERN-EuroBloodNet está a criar um directório de material educativo, esta acção pode ser realizada em conjunto)

Próximos passos

- Divulgar os resultados junto das associações nacionais, regionais e locais de drepanocitose e/ou outros doentes com drepanocitose, a fim de os informar sobre a criação da rede europeia de associações de drepanocitose.
- Eleger representantes nacionais.

ANNEX II

POSTER ECRD EU NET SCD PO

European
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complex diseases

Network
Hematological
Diseases (ERN EuroBloodNet)

ERN-EuroBloodNet: Establishment of the European Network of Sickle Cell Disease Patients Organizations

Pellegrini M* [1], Fenaux P [1], Gulbis B[2], Gutiérrez-Valle V[3], Mañú-Pereira MM[3]
[1]AP-HP, Hôpital Saint Louis, Paris, France, [2]Hôpital ERASME/LHUB-ULB, Brussels, Belgium, [3] University Hospital Vall d'Hebron - Vall d'Hebron Research Institute, Barcelona, Spain,

Sickle Cell Disease (SCD) is an inherited disorder of the red blood cells, traditionally endemic in African and Middle East countries. A lifelong chronic condition that can lead to disability or premature death in its severe forms.

SCD frequency has increased recently in Europe due to migration and mobility flows.

Patients' organizations (POs) presence at the national level is extremely varied from country to country. Many countries in Europe have no patients organization.

12 European countries reached

How to?

1. Identify SCD POs in Europe.

2. Invite POs to National Meetings .

3. Encourage, if not existing yet, to form a national association/federation/group.

4. Election of national representatives.

5. Establishment of the board in kick off meeting

6. Organization of Educational sessions for empowerment of patients advocates (ASCAT 2019 and 2020, UNESCO 2020)

National Meetings

- ✓ Milan, the 29th of March 2019
- ✓ Brussels, the 19th of June 2019 Informative Meeting
- ✓ Paris, the 29th of June 2019
- ✓ Madrid, gathering Spanish and Portuguese patients, 18th February 2020

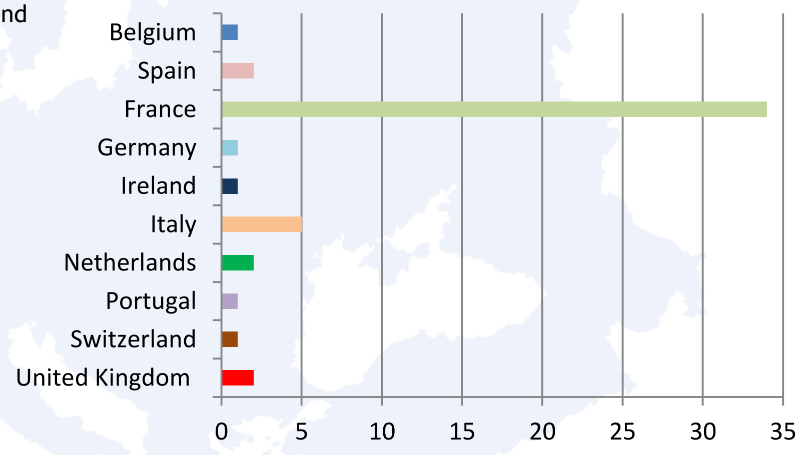
The European Reference Network on rare hematological diseases, ERN-EuroBloodNet, in collaboration with EURORDIS, is establishing the European Network of Sickle Cell Disease (SCD) Patients Organizations

Caratheristics

- A **bottom-up umbrella** network of national and local organizations of SCD patients' advocates
- Each Country is represented by **2 spokesmen** on behalf of all the national patients' organizations.
- Among all spokesmen a representative one would become **ePAG** for the ERN-EuroBloodNet.

50 Patients organisations reached

Patients Organizations reached



Number of POs per country reached by ERN-EuroBloodNet

A common unique voice for SCD patients advocates in Europe

European Reference Network
for rare or low prevalence complex diseases
Network Hematological Diseases (ERN EuroBloodNet)
Co-funded by the European Union
www.eurobloodnet.eu
EURORDIS RARE DISEASES EUROPE

ANNEX III

ASCAT 2019

European
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Network
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Diseases (ERN EuroBloodNet)

ASCAT
Annual Academy For
Sickle Cell And
Thalassaemia
Conference

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Hematological
Diseases (ERN EuroBloodNet)

AGENDA

Sickle Cell Disease Research Prioritisation Workshop at ASCAT 2019

Sunday, the 20th of October 2019

Informal Session
4 pm-7pm

VENUE:
Park Plaza London Waterloo, Hercules House, 6 Hercules Road, Lambeth, London, SE1 7DP.

- **4.00 - 4.05 pm** Round Table
- **4.05 - 4.10 pm** Welcome by ASCAT representative (Baba Inusa)
- **4.10 - 4.15 pm** Presentation of ERN-EuroBloodNet project dedicated to SCD (Mariangela Pellegrini)
- **4.15 - 4.20 pm** Being an expert patient. The importance of patient education (Mariangela Pellegrini)
- **4-20-5.40 pm** Patients expressing their experiences
- **5.40-7.00 pm** Patients expressing their priorities about Research and patient involvement in Research

7.15 pm Faculty Dinner

Monday, the 21st of October 2019

SCD Research Prioritisation Workshop
9 am – 1 pm

VENUE:
ASCAT congress, Venues, County Hall, Westminster Bridge, Belvedere road, London, SE1 7PB.

- **9 am – 10 am Welcome Coffee**
- **10-10.30 am** talks- What is patient and public involvement (PPI), how and why should patients get involved?
-

- **10.30-10.40 am** - What are your ideas about what topics for SCD research? Quiet time for each person to reflect on their own priorities for research (post-it notes will be made available for each person to write down their thoughts).
- **10.40-11.20 am** - Can we come up with a list as a group? We will form two groups to discuss ideas for SCD research, and each group will produce a list of topics.

- **11.20-11.40 am** , coffee break

- **11.40-11.50 am** Putting the 2 lists together. Each table will share their ideas for research topics, and we will agree a complete list as a whole group
- **11.50-12.20 am** What do we want at the top of the list? We will break up again into 2 different groups and each group will come up with a 'Top 10' of SCD research topics.
- **12.20-12.40 am** Can we all agree on a common Top 10? The whole group gets back together to agree a Top ten list (consensus phase) to be presented to the conference.
- **12.40-13.00 am** Prepare presentation. Help and advice available for making slides and planning what will be said in the feedback to the conference.

Plenary ASCAT Congress
4 pm

- **4.00-4.20 pm** Patient group presents "Top 10" list of topics that patients think are important for research into SCD to whole of ASCAT audience.

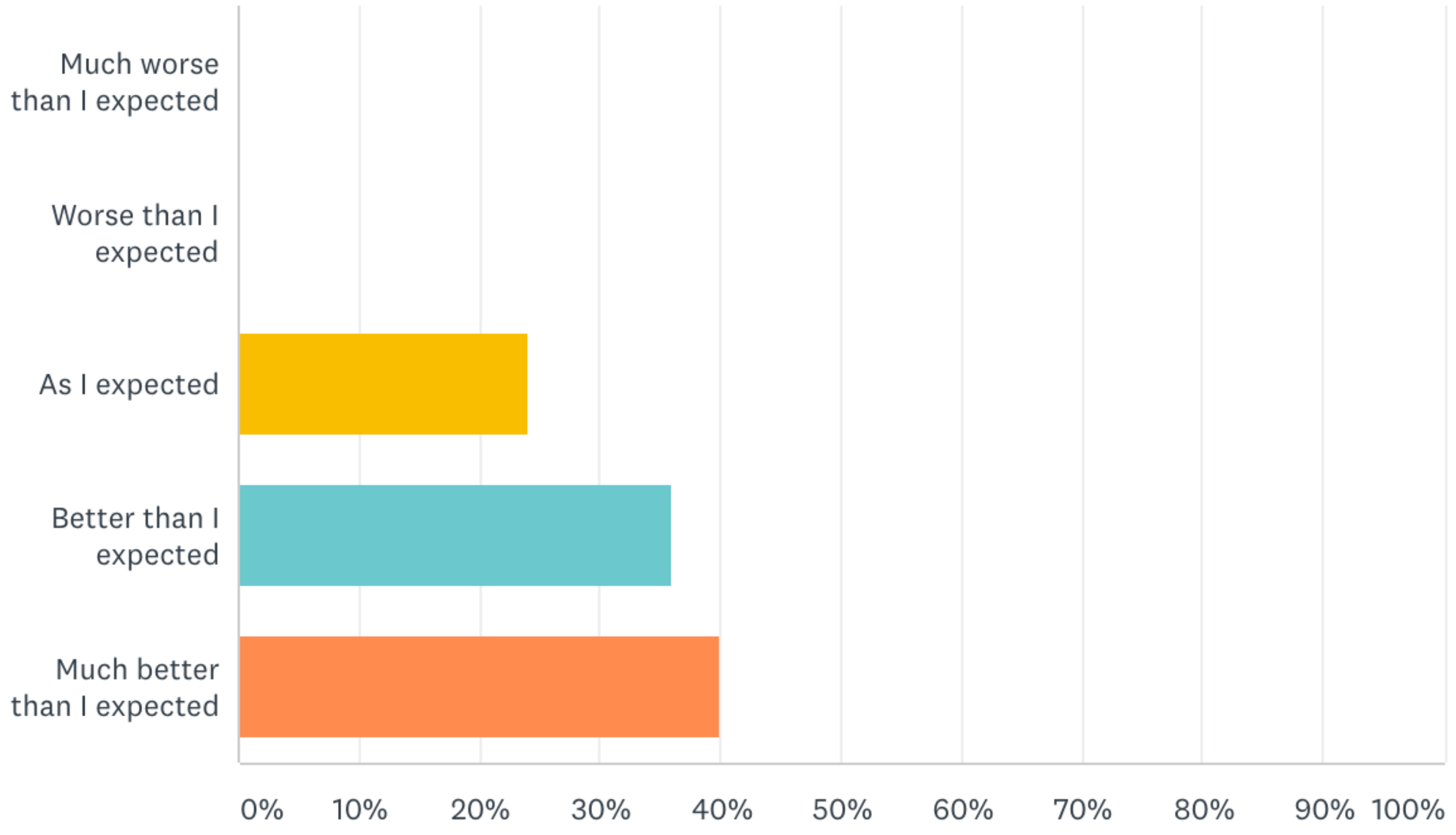
ASCAT feedback from patient workshop

ASCAT Oct 2019

Feedback Jan 2020

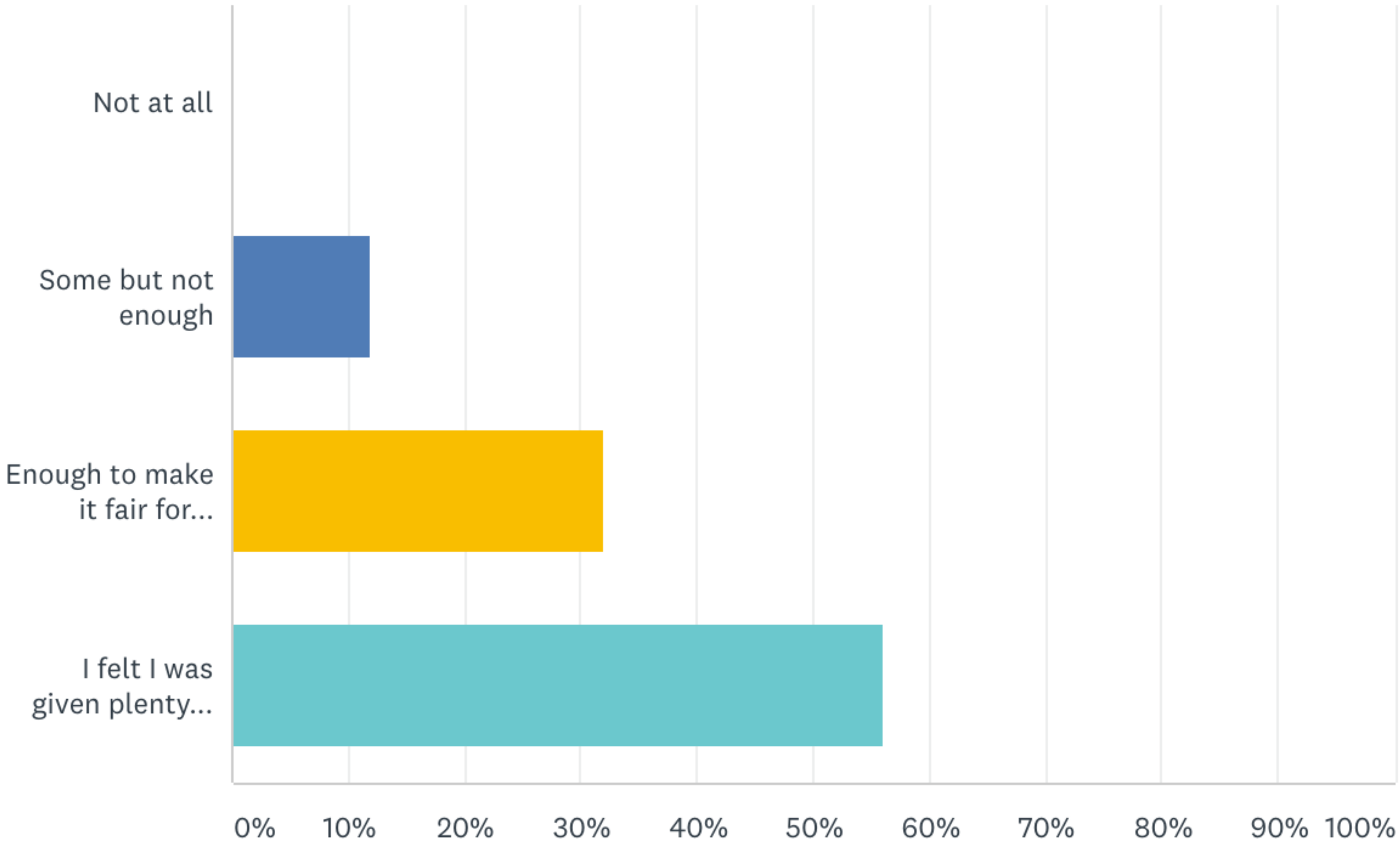
How did the day compare to what you were expecting:

Answered: 25 Skipped: 1



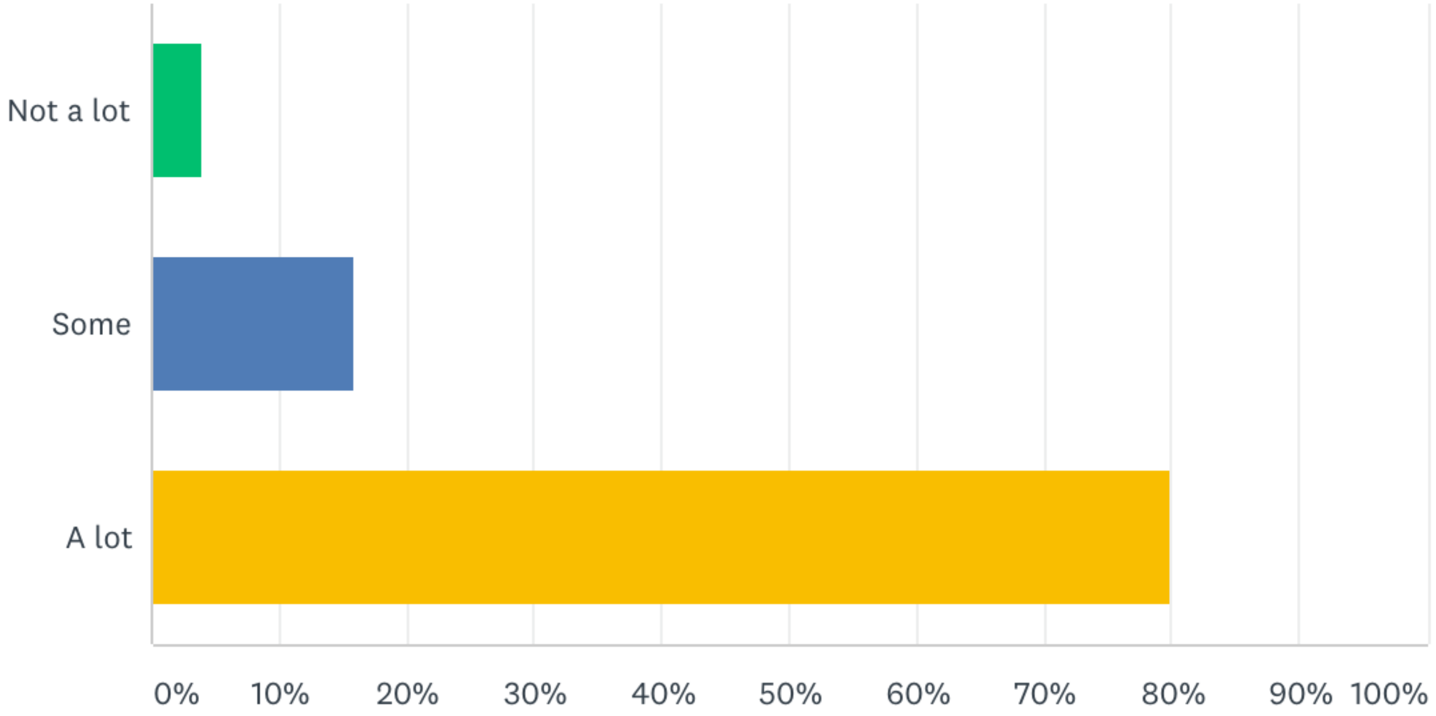
To what extent did you feel you were given a chance to give your opinion:

Answered: 25   Skipped: 1



How much do you feel you gained by the experience?

Answered: 25   Skipped: 1



How well did you think the workshop was run?

Answered: 24 Skipped: 2

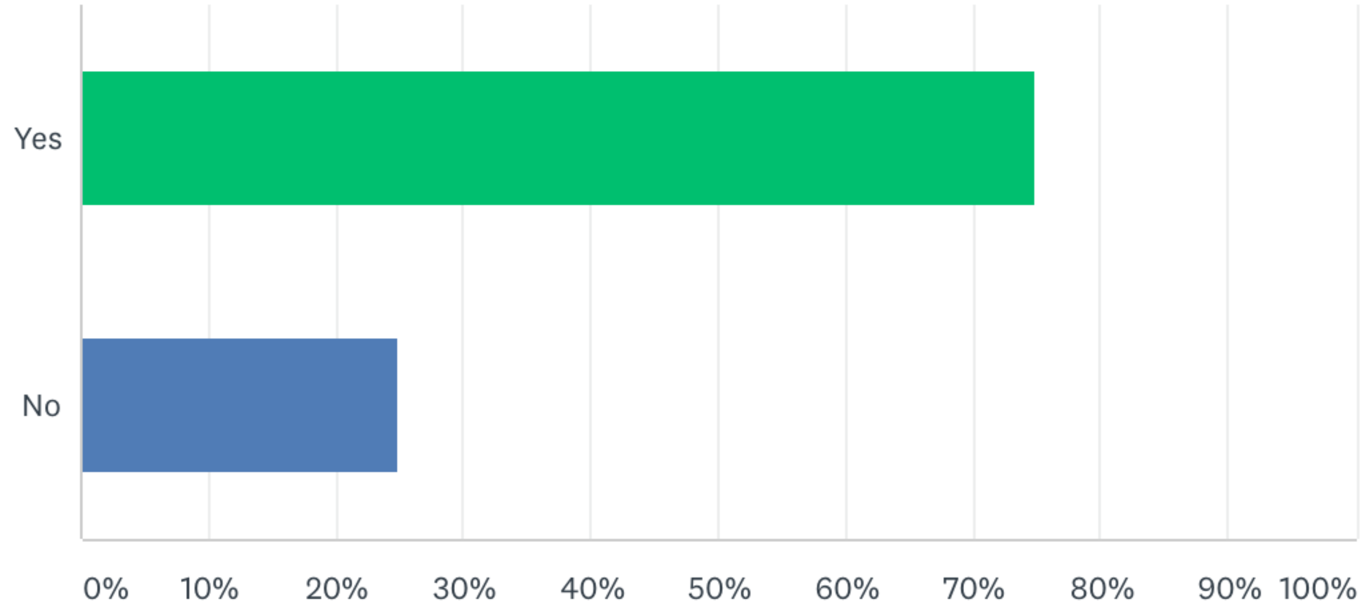
4.5★

average rating

	1	2	3	4	5	TOTAL	WEIGHTED AVERAGE
☆	0.00% 0	0.00% 0	8.33% 2	33.33% 8	58.33% 14	24	4.50

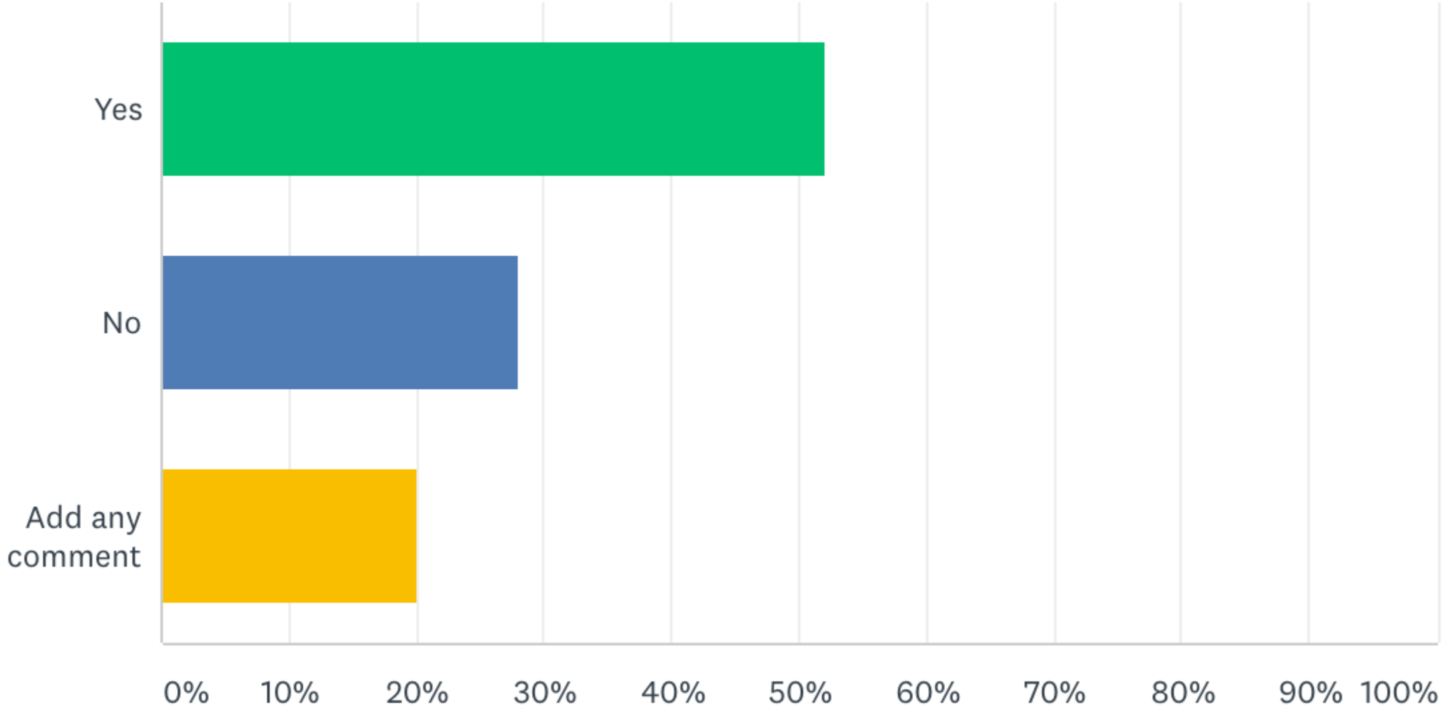
Did you feel the organisers paid enough attention to those who did not speak English as their first language?

Answered: 24   Skipped: 2

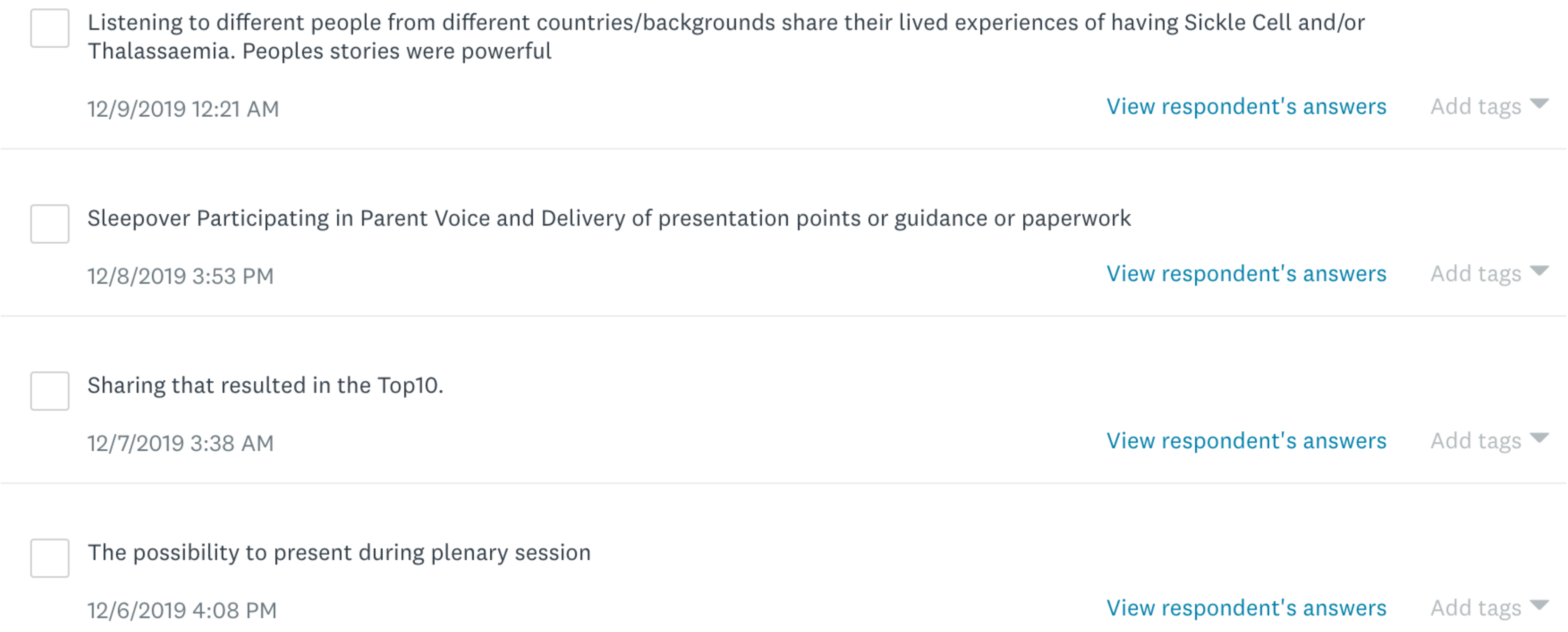


Did you feel you had enough information before you came to the workshop?

Answered: 25   Skipped: 1



What was the most powerful aspect of the workshop for you? (1)



☐ Listening to different people from different countries/backgrounds share their lived experiences of having Sickle Cell and/or Thalassaemia. Peoples stories were powerful

12/9/2019 12:21 AM

☐ Sleepover Participating in Parent Voice and Delivery of presentation points or guidance or paperwork

12/8/2019 3:53 PM

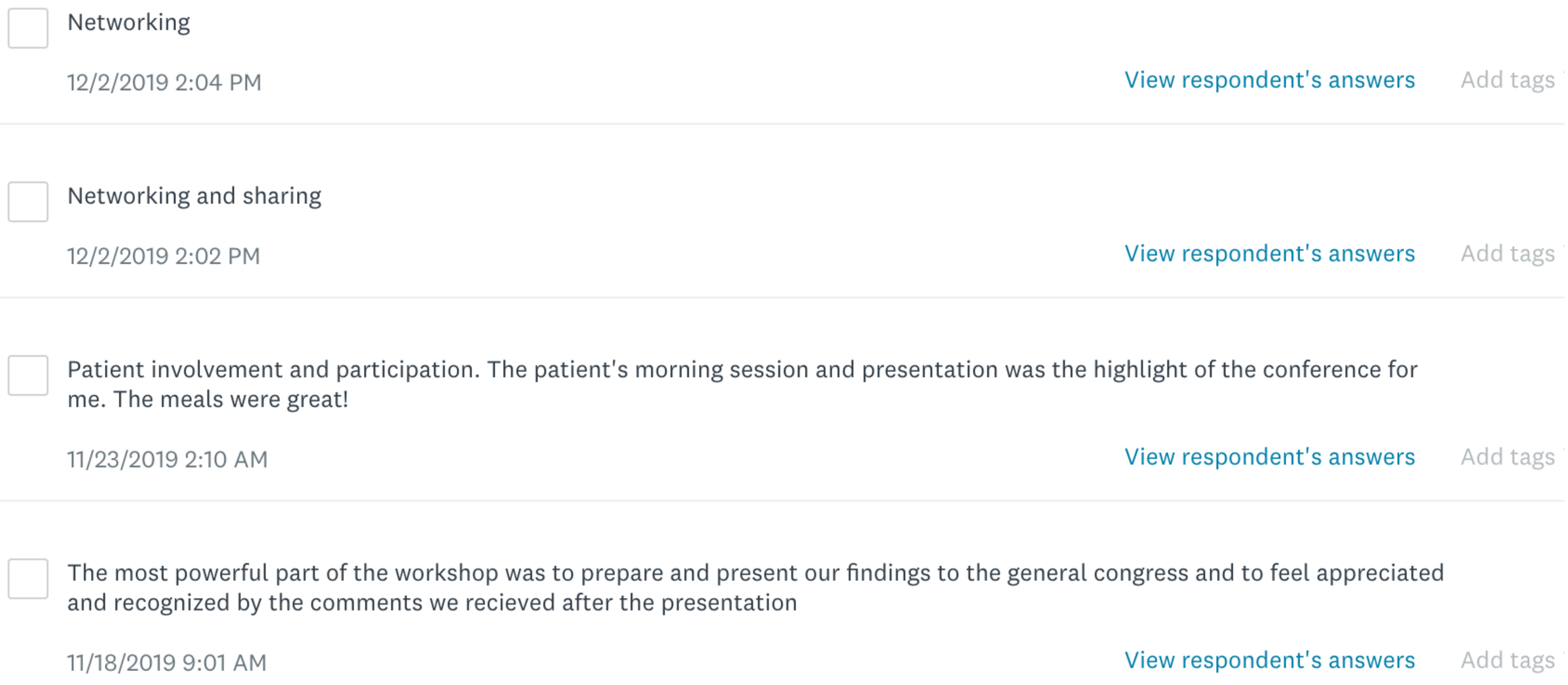
☐ Sharing that resulted in the Top10.

12/7/2019 3:38 AM

☐ The possibility to present during plenary session

12/6/2019 4:08 PM

What was the most powerful aspect of the workshop for you? (2)



☐ Networking

12/2/2019 2:04 PM

☐ Networking and sharing

12/2/2019 2:02 PM

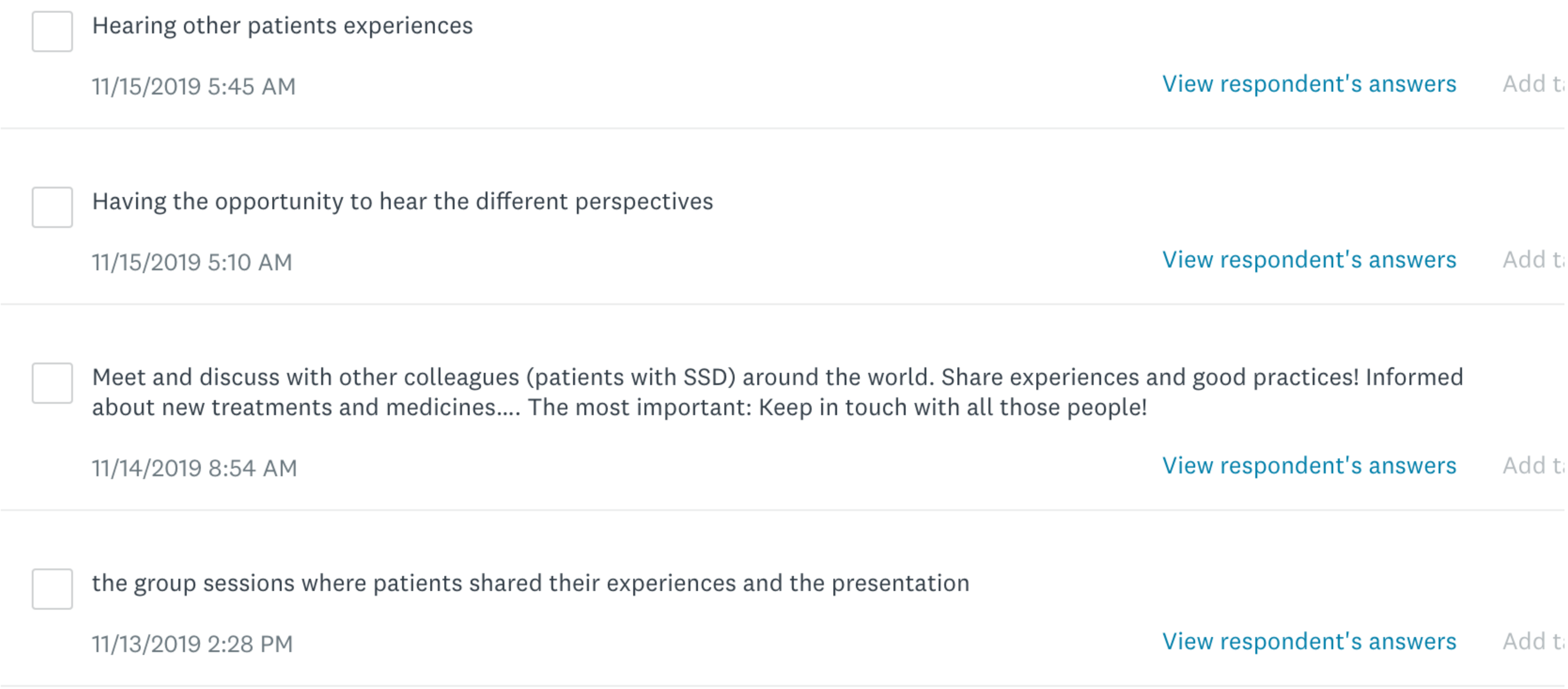
☐ Patient involvement and participation. The patient's morning session and presentation was the highlight of the conference for me. The meals were great!

11/23/2019 2:10 AM

☐ The most powerful part of the workshop was to prepare and present our findings to the general congress and to feel appreciated and recognized by the comments we recieved after the presentation

11/18/2019 9:01 AM

What was the most powerful aspect of the workshop for you? (3)



☐ Hearing other patients experiences

11/15/2019 5:45 AM

☐ Having the opportunity to hear the different perspectives

11/15/2019 5:10 AM

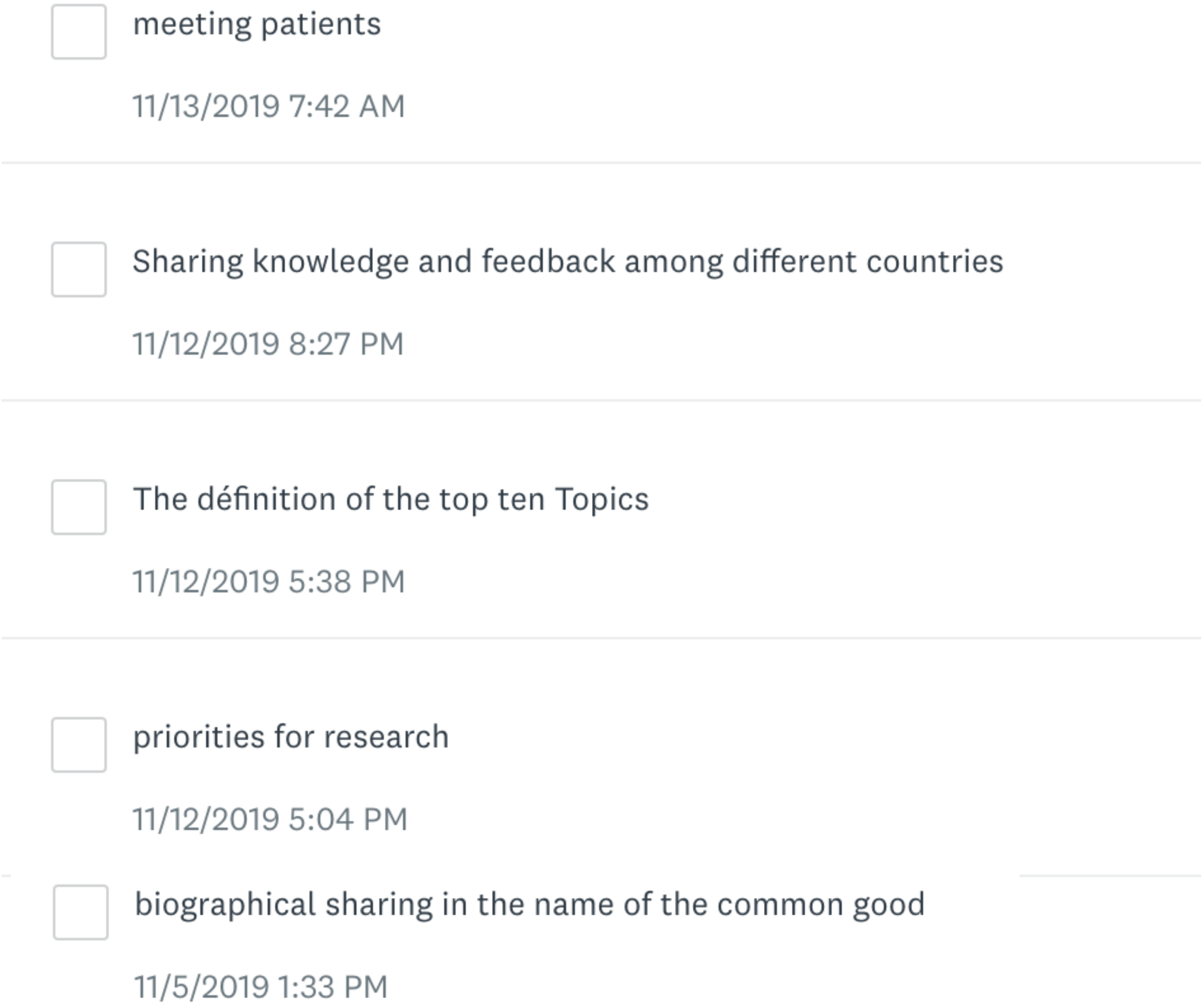
☐ Meet and discuss with other colleagues (patients with SSD) around the world. Share experiences and good practices! Informed about new treatments and medicines.... The most important: Keep in touch with all those people!

11/14/2019 8:54 AM

☐ the group sessions where patients shared their experiences and the presentation

11/13/2019 2:28 PM

What was the most powerful aspect of the workshop for you? (4)



☐ meeting patients

11/13/2019 7:42 AM

☐ Sharing knowledge and feedback among different countries

11/12/2019 8:27 PM

☐ The définition of the top ten Topics

11/12/2019 5:38 PM

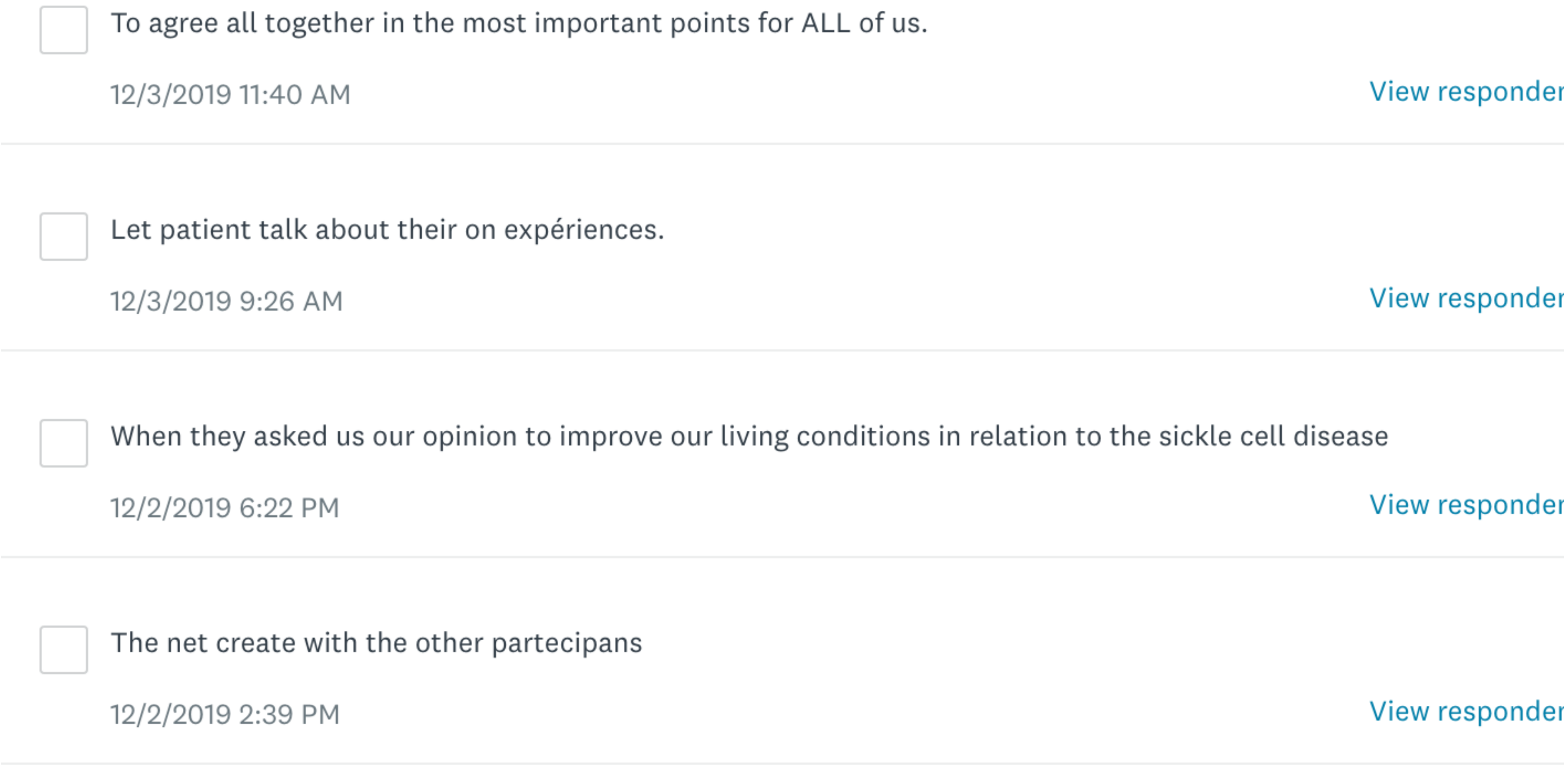
☐ priorities for research

11/12/2019 5:04 PM

☐ biographical sharing in the name of the common good

11/5/2019 1:33 PM

What was the most powerful aspect of the workshop for you? (5)



☐ To agree all together in the most important points for ALL of us.

12/3/2019 11:40 AM

☐ Let patient talk about their on expériences.

12/3/2019 9:26 AM

☐ When they asked us our opinion to improve our living conditions in relation to the sickle cell disease

12/2/2019 6:22 PM

☐ The net create with the other partecipans

12/2/2019 2:39 PM

What was the most powerful aspect of the workshop for you? (6)

☐ The whole experience

12/6/2019 3:23 PM

☐ Patients presence and discussion

12/5/2019 10:17 AM

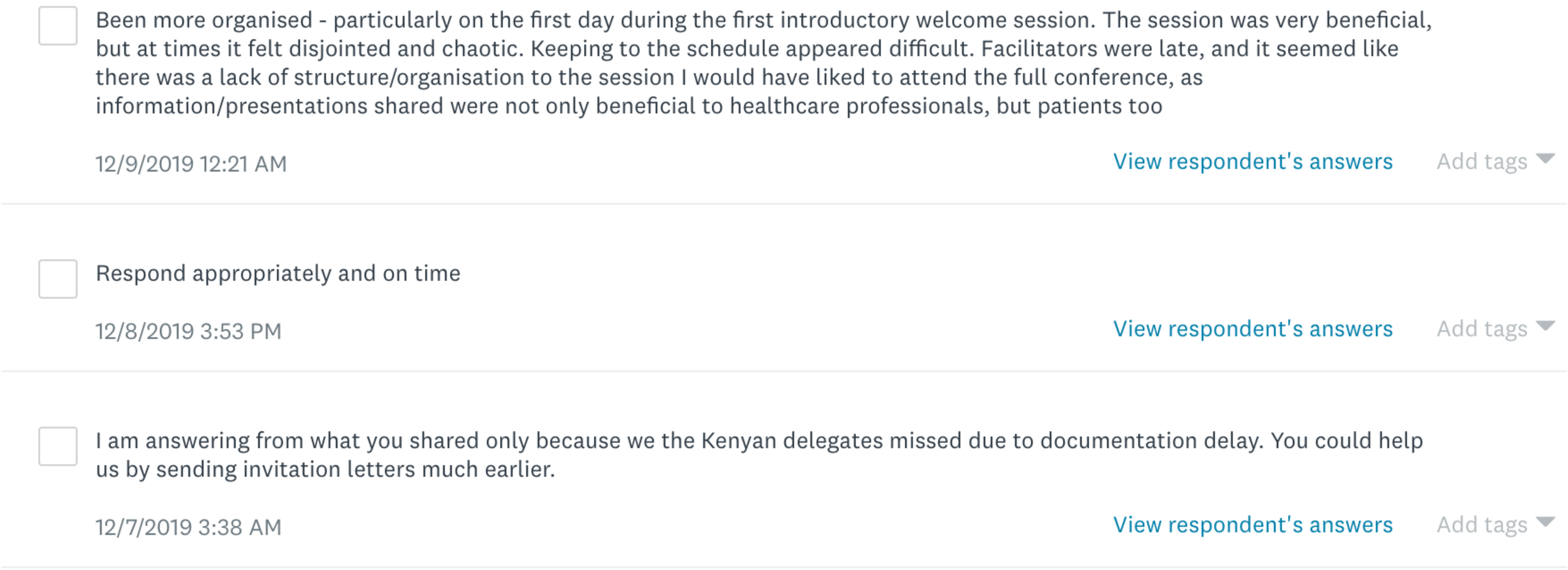
☐ The organisation

12/4/2019 7:05 AM

☐ Patient's personal experiences

12/3/2019 12:16 PM

What do you think we could have done better? (1)



☐ Been more organised - particularly on the first day during the first introductory welcome session. The session was very beneficial, but at times it felt disjointed and chaotic. Keeping to the schedule appeared difficult. Facilitators were late, and it seemed like there was a lack of structure/organisation to the session I would have liked to attend the full conference, as information/presentations shared were not only beneficial to healthcare professionals, but patients too

12/9/2019 12:21 AM

☐ Respond appropriately and on time

12/8/2019 3:53 PM

☐ I am answering from what you shared only because we the Kenyan delegates missed due to documentation delay. You could help us by sending invitation letters much earlier.

12/7/2019 3:38 AM

What do you think we could have done better? (2)

☐ The first day was not well organized and a little bit messy. It looked like a support group. UK was over representanted, it did not give enough space for other countries representatives

12/6/2019 4:08 PM

☐ NA

12/6/2019 3:23 PM

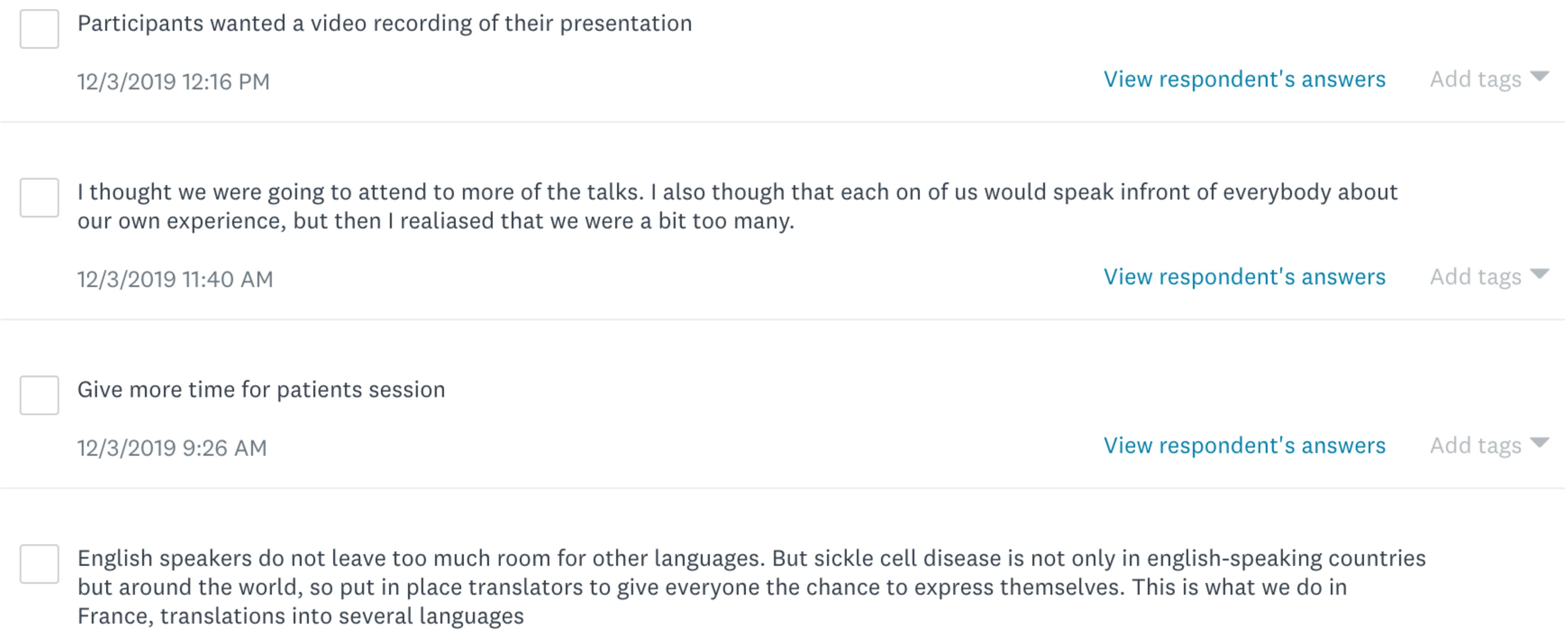
☐ Allow all persons to talk

12/5/2019 10:17 AM

☐ More planning before questions

12/4/2019 7:05 AM

What do you think we could have done better? (3)



☐ Participants wanted a video recording of their presentation

12/3/2019 12:16 PM

☐ I thought we were going to attend to more of the talks. I also though that each on of us would speak infront of everybody about our own experience, but then I realiased that we were a bit too many.

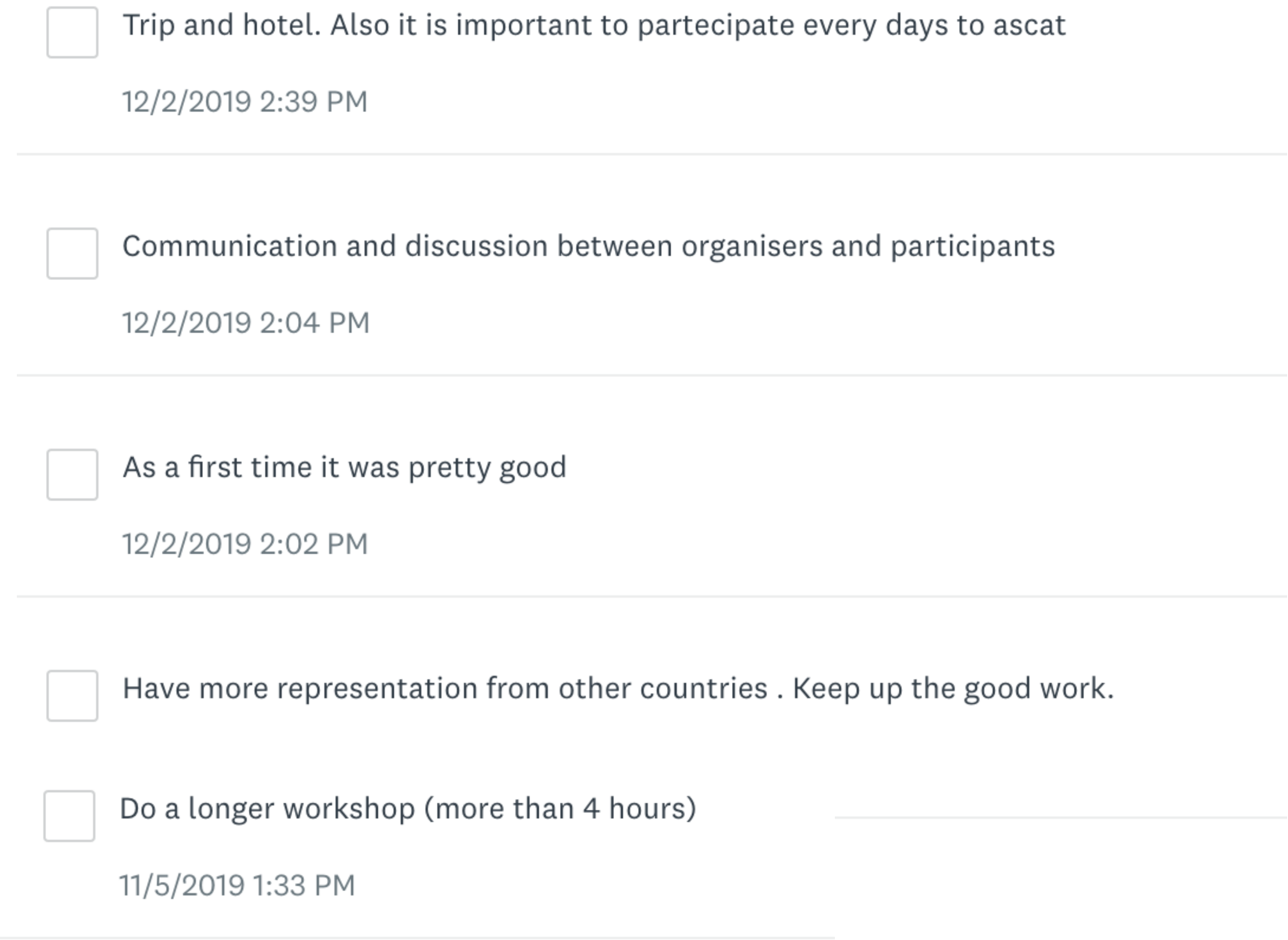
12/3/2019 11:40 AM

☐ Give more time for patients session

12/3/2019 9:26 AM

☐ English speakers do not leave too much room for other languages. But sickle cell disease is not only in english-speaking countries but around the world, so put in place translators to give everyone the chance to express themselves. This is what we do in France, translations into several languages

What do you think we could have done better? (4)



☐ Trip and hotel. Also it is important to partecipate every days to ascat

12/2/2019 2:39 PM

☐ Communication and discussion between organisers and participants

12/2/2019 2:04 PM

☐ As a first time it was pretty good

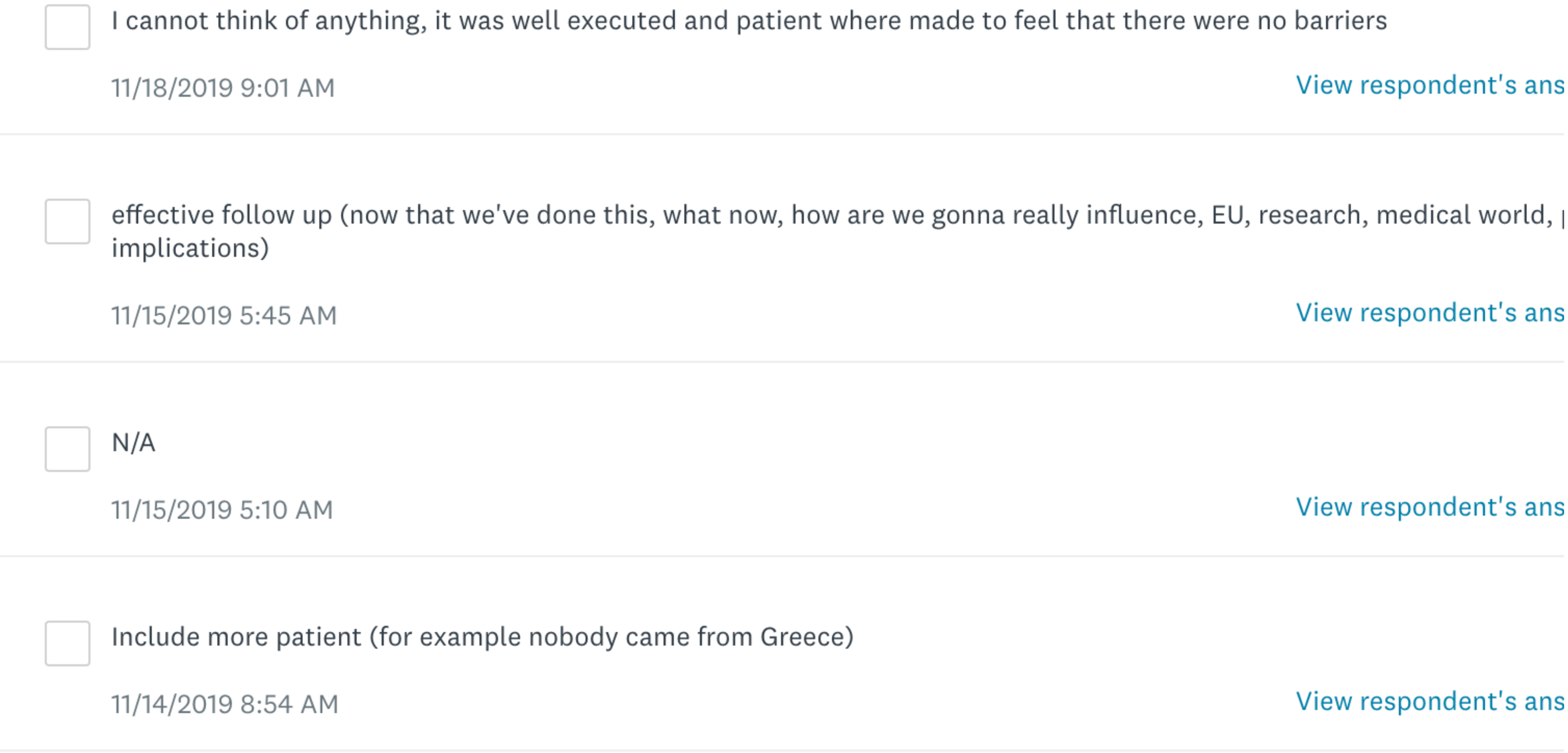
12/2/2019 2:02 PM

☐ Have more representation from other countries . Keep up the good work.

☐ Do a longer workshop (more than 4 hours)

11/5/2019 1:33 PM

What do you think we could have done better? (5)



☐ I cannot think of anything, it was well executed and patient where made to feel that there were no barriers

11/18/2019 9:01 AM
View respondent's ans

☐ effective follow up (now that we've done this, what now, how are we gonna really influence, EU, research, medical world,
implications)

11/15/2019 5:45 AM
View respondent's ans

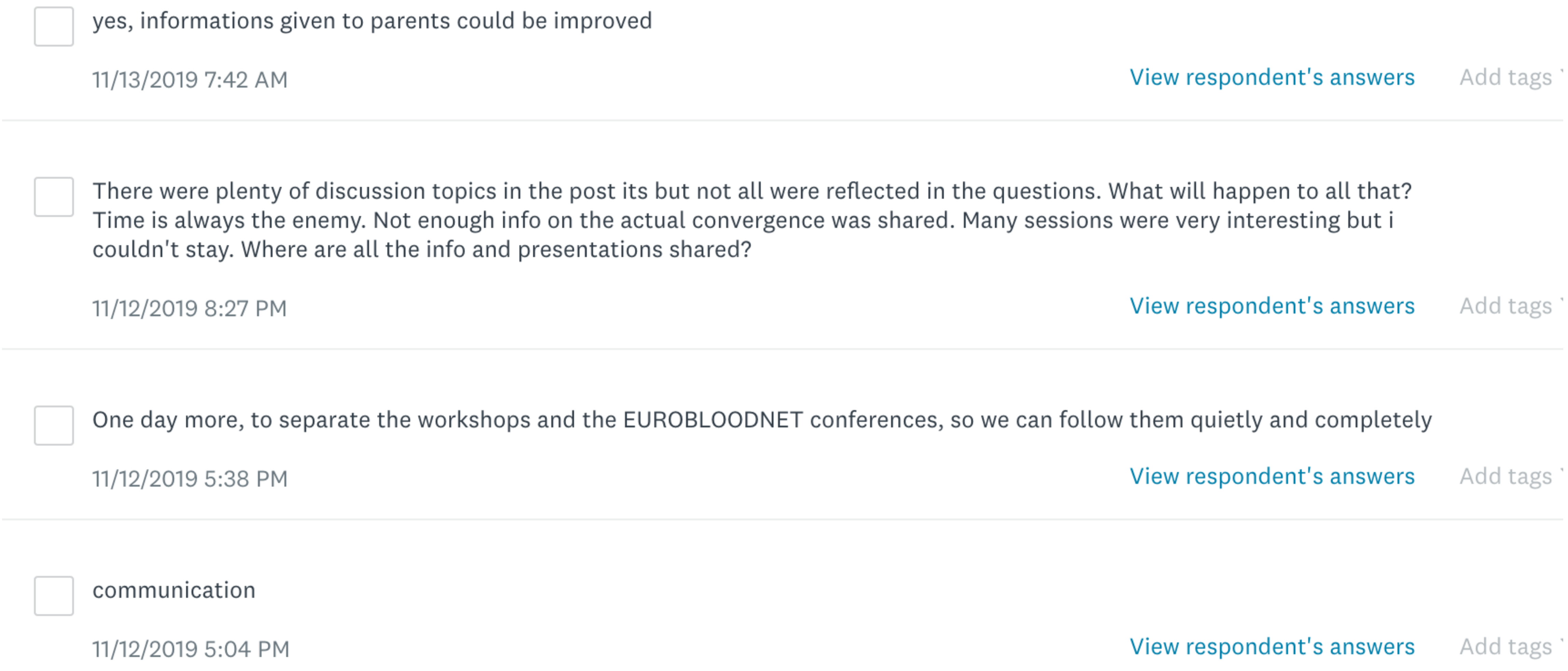
☐ N/A

11/15/2019 5:10 AM
View respondent's ans

☐ Include more patient (for example nobody came from Greece)

11/14/2019 8:54 AM
View respondent's ans

What do you think we could have done better? (6)



☐ yes, informations given to parents could be improved

11/13/2019 7:42 AM

☐ There were plenty of discussion topics in the post its but not all were reflected in the questions. What will happen to all that? Time is always the enemy. Not enough info on the actual convergence was shared. Many sessions were very interesting but i couldn't stay. Where are all the info and presentations shared?

11/12/2019 8:27 PM

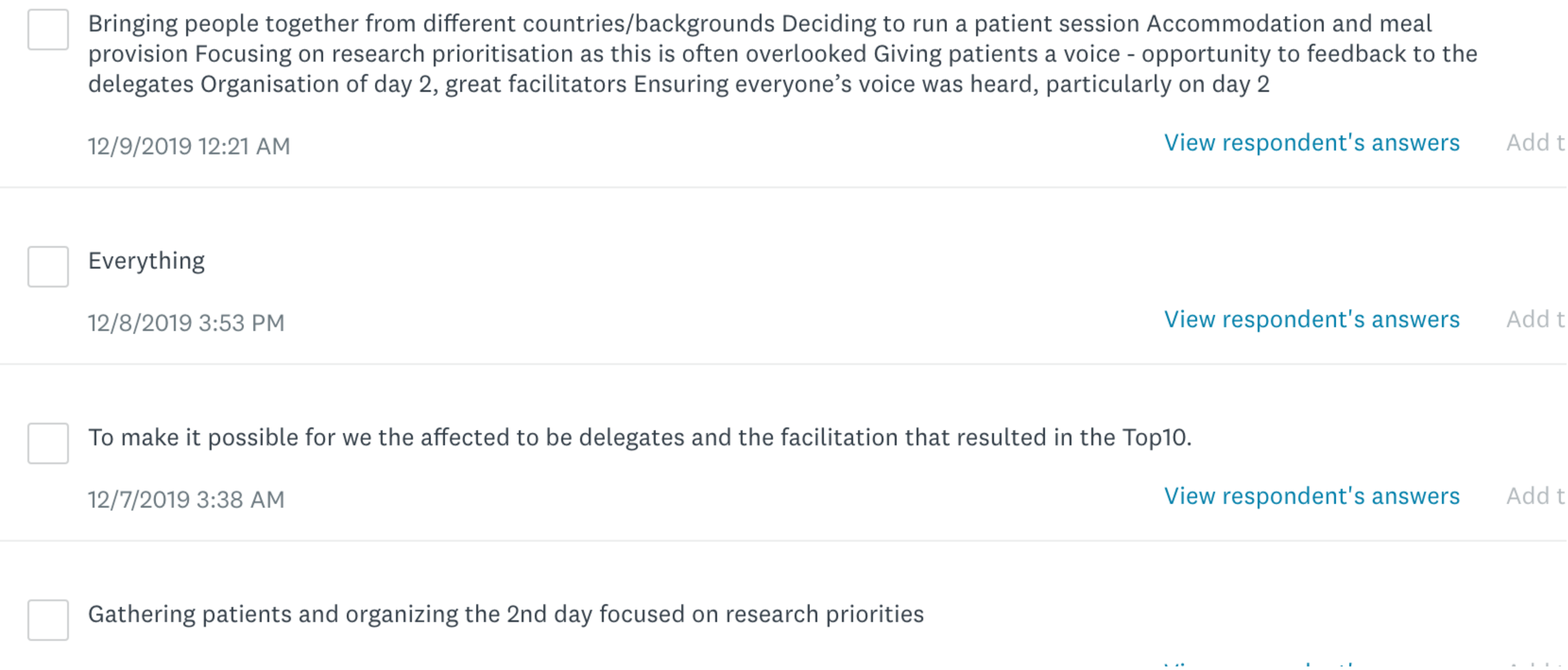
☐ One day more, to separate the workshops and the EUROBLOODNET conferences, so we can follow them quietly and completely

11/12/2019 5:38 PM

☐ communication

11/12/2019 5:04 PM

What do you think we did well? (1)



☐ Bringing people together from different countries/backgrounds Deciding to run a patient session Accommodation and meal provision Focusing on research prioritisation as this is often overlooked Giving patients a voice - opportunity to feedback to the delegates Organisation of day 2, great facilitators Ensuring everyone’s voice was heard, particularly on day 2

12/9/2019 12:21 AM

☐ Everything

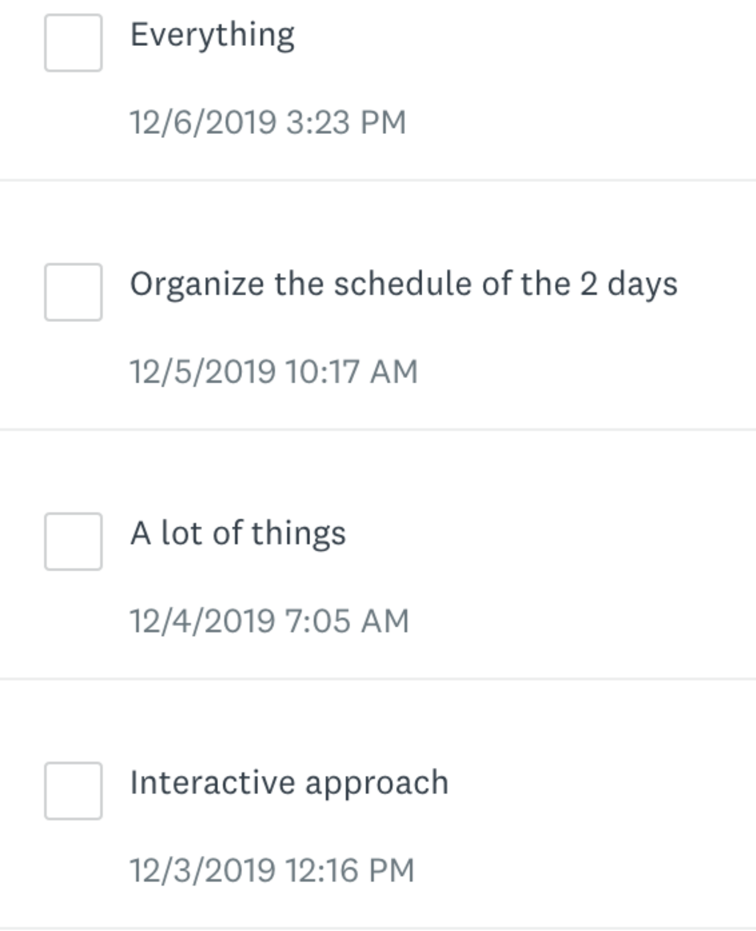
12/8/2019 3:53 PM

☐ To make it possible for we the affected to be delegates and the facilitation that resulted in the Top10.

12/7/2019 3:38 AM

☐ Gathering patients and organizing the 2nd day focused on research priorities

What do you think we did well? (2)



☐ Everything

12/6/2019 3:23 PM

☐ Organize the schedule of the 2 days

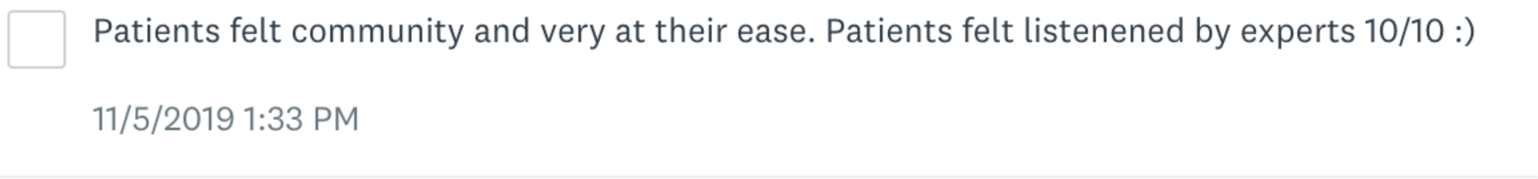
12/5/2019 10:17 AM

☐ A lot of things

12/4/2019 7:05 AM

☐ Interactive approach

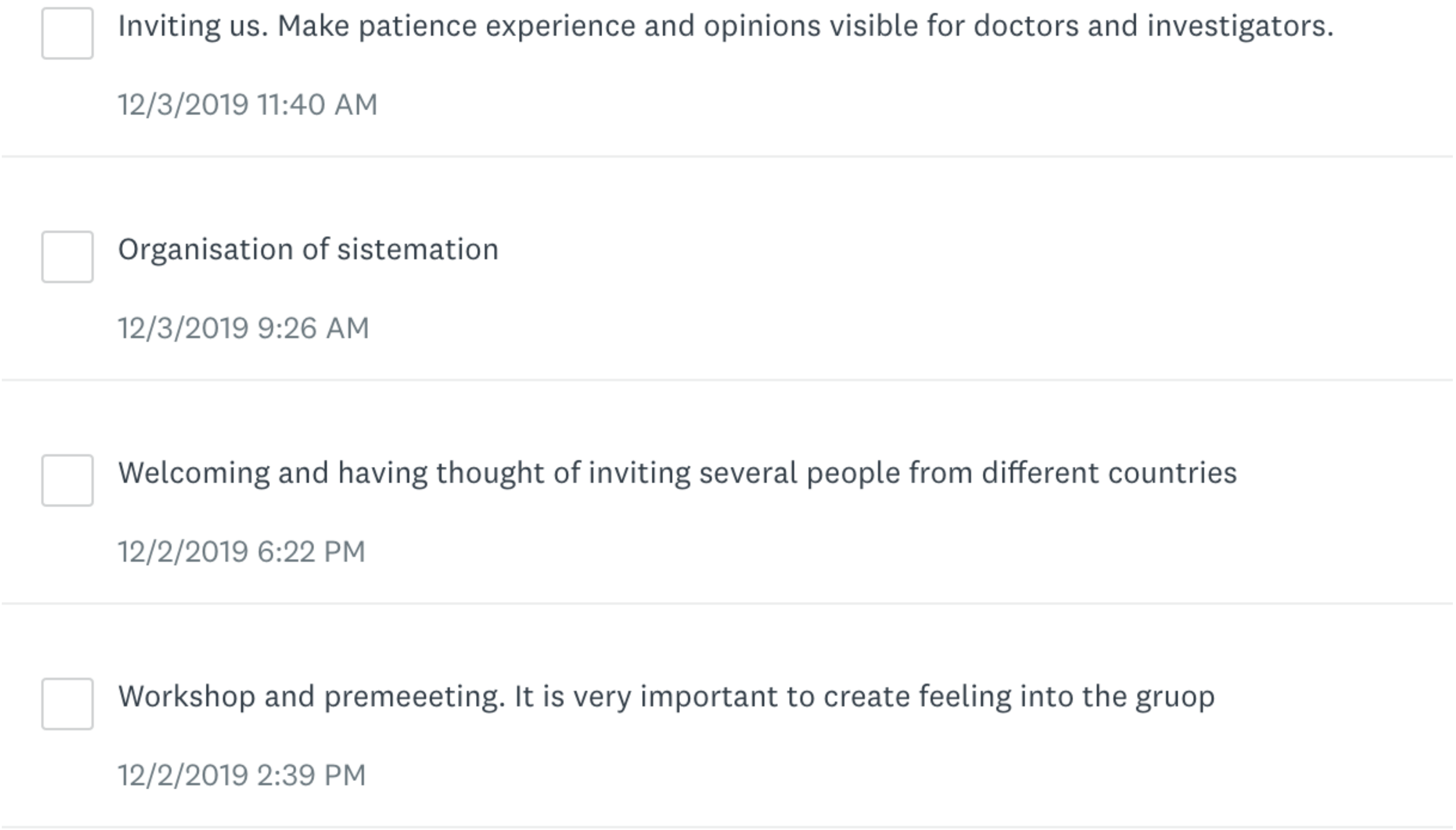
12/3/2019 12:16 PM



☐ Patients felt community and very at their ease. Patients felt listenened by experts 10/10 :)

11/5/2019 1:33 PM

What do you think we did well? (3)



☐ Inviting us. Make patience experience and opinions visible for doctors and investigators.

12/3/2019 11:40 AM

☐ Organisation of sistemation

12/3/2019 9:26 AM

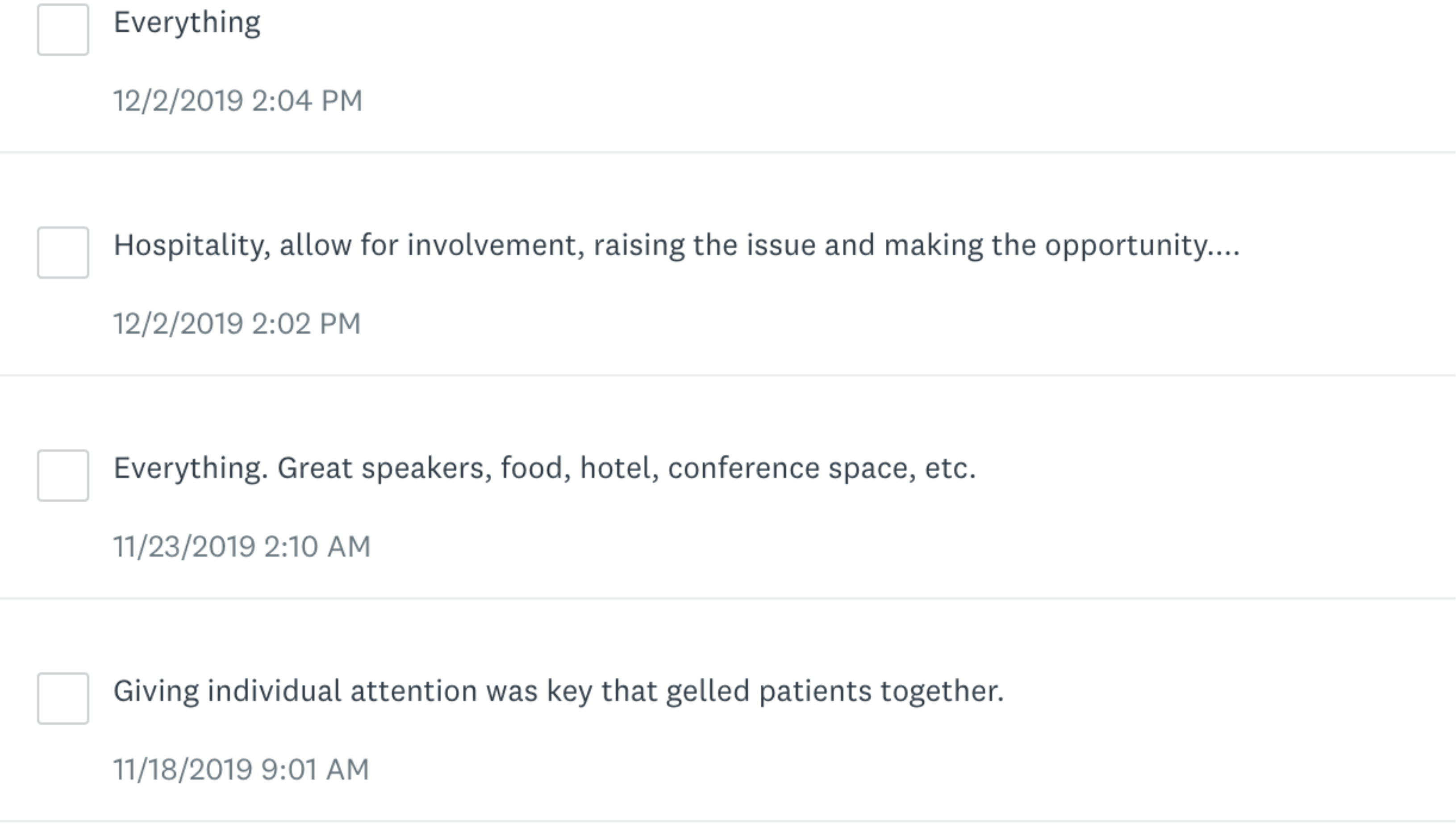
☐ Welcoming and having thought of inviting several people from different countries

12/2/2019 6:22 PM

☐ Workshop and premeeeting. It is very important to create feeling into the gruop

12/2/2019 2:39 PM

What do you think we did well? (4)



☐ Everything

12/2/2019 2:04 PM

☐ Hospitality, allow for involvement, raising the issue and making the opportunity....

12/2/2019 2:02 PM

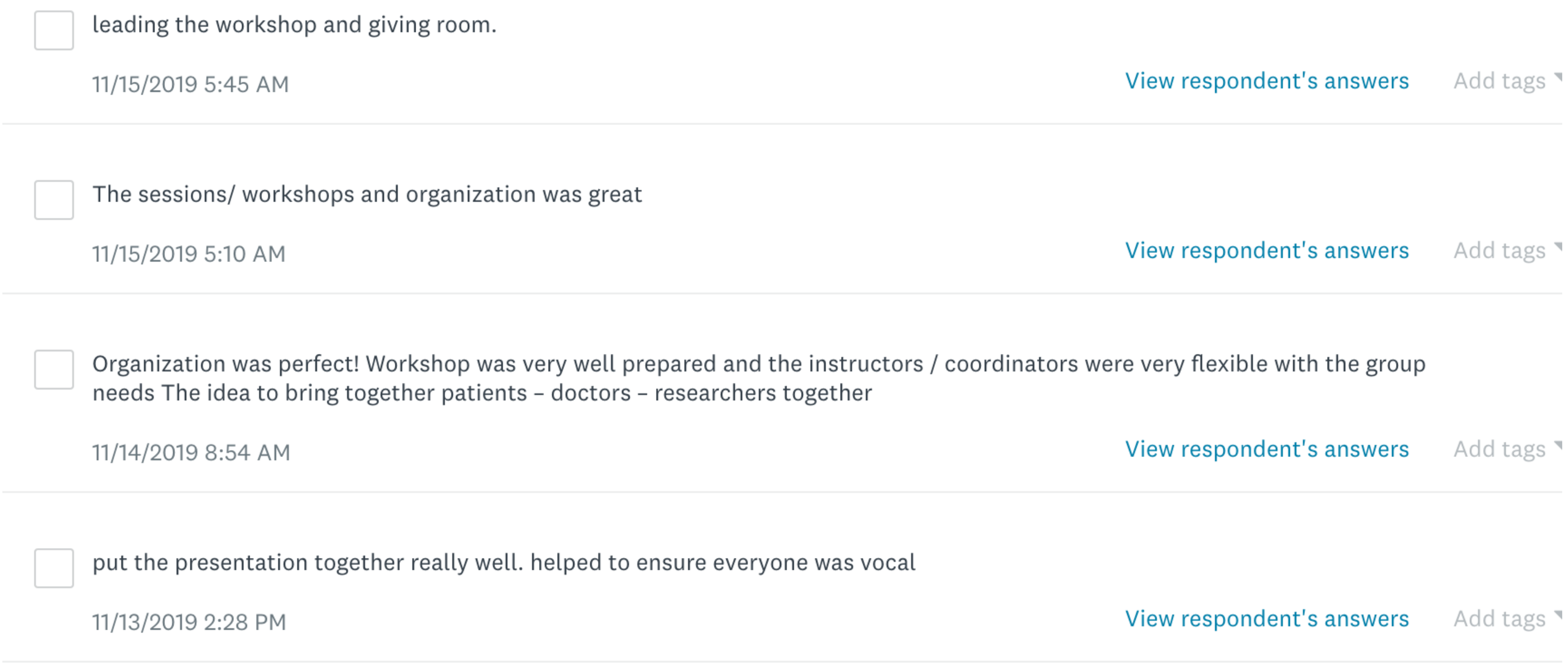
☐ Everything. Great speakers, food, hotel, conference space, etc.

11/23/2019 2:10 AM

☐ Giving individual attention was key that gelled patients together.

11/18/2019 9:01 AM

What do you think we did well? (5)



☐ leading the workshop and giving room.

11/15/2019 5:45 AM

☐ The sessions/ workshops and organization was great

11/15/2019 5:10 AM

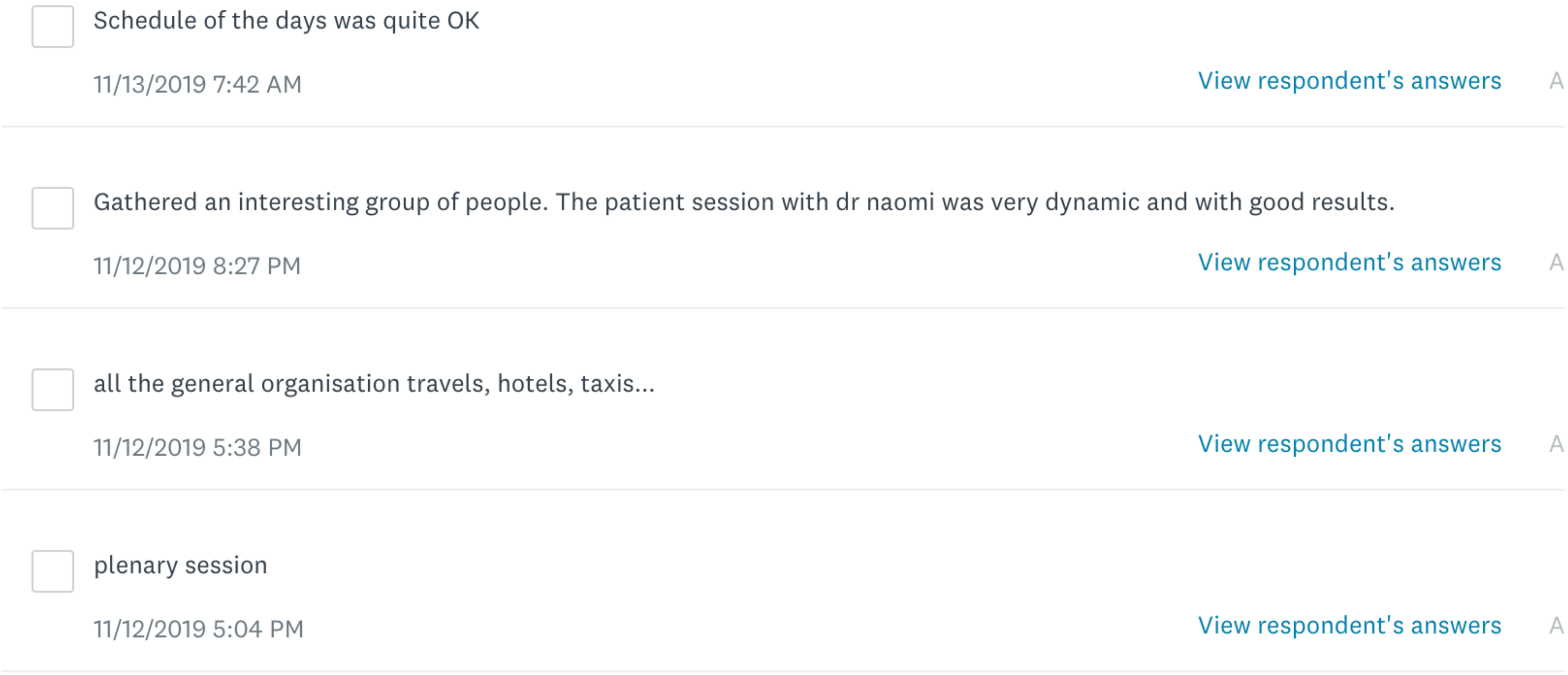
☐ Organization was perfect! Workshop was very well prepared and the instructors / coordinators were very flexible with the group needs The idea to bring together patients – doctors – researchers together

11/14/2019 8:54 AM

☐ put the presentation together really well. helped to ensure everyone was vocal

11/13/2019 2:28 PM

What do you think we did well? (6)



☐ Schedule of the days was quite OK

11/13/2019 7:42 AM

☐ Gathered an interesting group of people. The patient session with dr naomi was very dynamic and with good results.

11/12/2019 8:27 PM

☐ all the general organisation travels, hotels, taxis...

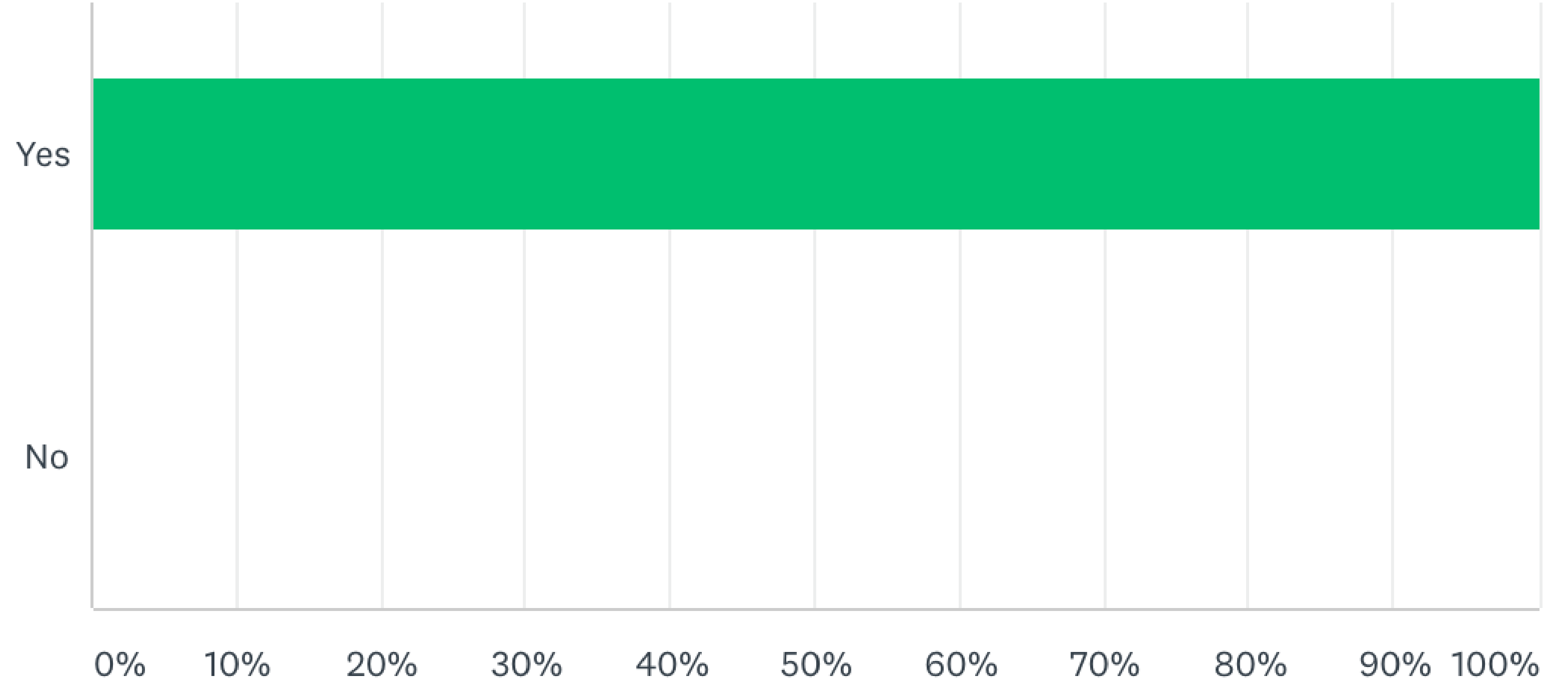
11/12/2019 5:38 PM

☐ plenary session

11/12/2019 5:04 PM

Would you like to stay involved with this group?

Answered: 26   Skipped: 0



ANNEX IV

ASCAT EHA

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Hematological
Diseases (ERN EuroBloodNet)

EUROPEAN
HEMATOLOGY
ASSOCIATION

Pragmatic Approach to empowering patients with SCD: Top 10 Research Priorities from the European EuroBloodNet/ASCAT Network of SCD Patients

M. PELLEGRINI [1], R. COLOMBATTI[2], C. GILMOUR-HAMILTON [3], S. HAYES[4], M. DE MONTALEMBERT[5], B. GULBIS[6], M. M. MAÑU PEREIRA[7], B. INUSA[8], K. A. ANIE[9] , V. GUTIERREZ-VALLE[7], O. AGBETU [10], D. ADJAGBA [10], Y. ADJIBI[10], A.ATHANASIOU,[10] I. B. CHUKWUDOZIE[10], F. DASAOLU[10], M. SANTOS FREIRE[10], A. KEKEH[10] and OTHER SCD PATIENTS REPRESENTATIVES FROM THE EUROBLOONET/ASCAT NETWORK, N. ROY [3]

[1]Service d'hématologie senior, Hôpital Saint-Louis, Paris, France. [2]Clinic of Pediatric Hematology-Oncology, Azienda Ospedaliera-Università di Padova, Padova, Italy. [3]Department of Haematology, Oxford University Hospitals NHS Foundation Trust. [4]Department of Haematology, Oxford University Hospital NHS Trust, Oxford, United Kingdom. [5]Department of Pediatric Surgery, Hôpital Universitaire Necker-Enfants malades, Paris, France. [6]Laboratoire Hospitalier Universitaire de Bruxelles, Hôpital Erasme-CUB, Bruxelles, Belgium. [7]Vall d'Hebron, Research Institute, University Hospital Vall d'Hebron, Barcelona, Spain. [8]Paediatric Haematology Department, Guy's & St Thomas' NHS Foundation Trust, London, United Kingdom. [9]Haematology and Sickle Cell Centre, London North West University Healthcare NHS Trust, London, United Kingdom. [10]EuroBloodNet/ASCAT SCD Patients' representatives Network.

INTRODUCTION

Sickle Cell Disease (SCD) is a lifelong inherited red blood cell disorder which can lead to disability or even premature death in its severe forms. People living with SCD in Europe mainly belong to social minorities and are more isolated, have a lower level of health education and poorer adherence and disease management compared with patients with other hematologic diseases.

With the creation of rare disease networks, the EU has provided a requirement and the means of empowering patients and enhancing their involvement in their disease management. ERN-EuroBloodNet promotes educational activities addressed at people living with SCD in order to encourage their training and develop their skills as advocate and expert patients able to create a community whose common aim is to address patients' unmet needs.

OBJECTIVES

The SCD Research Prioritisation Workshop at the Annual Scientific Conference on Sickle Cell and Thalassaemia (ASCAT) was a joint project of the ERN-EuroBloodNet, ASCAT members and the patient engagement group Oxford Blood Group. Its aims were to introduce Patient Involvement in Research (PPI) to people living with SCD, guide them through a research prioritization exercise, and assess their expectations and feedback.

METHODS

This patient forum participated in a workshop to discuss research priorities. Drawing on their own experience of SCD, the group were asked to write down issues that they felt demanded greater attention from research- a single word, an experience, or a fully formed research question.

The ideas were written on post-it notes and pinned on the wall of the conference room. The facilitators grouped the individual notes into 50 individual themes. The group then collectively discussed each theme to arrive at a number of research questions or topics, resulting in 42 unique research questions. The group were then invited to vote for their top 10- each individual was given 10 post-it notes, representing a hypothetical "$100,000,000" to spend on research. They were asked: "If you were allocating this money for research projects, how would you spend it?" The results of this process were counted to arrive at the Top 10 research topics for SCD. Finally, the group created a presentation and presented it to the conference in the afternoon plenary session.

FIGURE 1

SCD TOP TEN RESEARCH TOPICS

Lived experience and the SCD research agenda

OUTCOMES OF A PRIORITY SETTING WORKSHOP WITH SCD PATIENTS AND CARERS, 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 hospitalisaton?
5. How does staff diversity or lack of diversity affect the way patients experience racism, discrimination and stigma?
6. How do we harness the political agendas to improve care for sickle cell disease?
7. How can we optimise pregnancy for mother and baby in sickle cell disease?
8. What complementary therapies work in sickle cell disease, including CBD oil and cannabis?
9. How do we manage chronic pain?
10. What is the impact of a comprehensive care team on outcomes for people living with sickle cell disease?

CONTACT INFORMATION

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- Noémi Roy, noemi@romahi.com

RESULTS

The workshop methodology was adapted from the James Lind Alliance to gain a valid consensus and moderated by three experienced facilitators. SCD patients were identified through National SCD Networks, ASCAT members and EHA ERN-EuroBloodNet representatives. A group of 28 people from 12 countries were invited to ASCAT 2019 to create a patient forum to share experiences, build cross-border partnerships and discuss good practice for local patient support groups.

The Top 10 Research Topics which were devised and selected by the group are shown in Figure 1. While the top priority to them is to fin a universally available cure, quality of life, social and political issues were high on the agenda, and represent topics that the research community should focus on in addition to biological issues traditionally funded.

CONCLUSIONS

The workshop allowed patients to experience Patient and Public Involvement and develop a meaningful list of questions which they would like research efforts to address. In what we believe is the first meeting of European patients with SCD, we show that there is common ground of unmet need for patients across the EU. In this unique output, the patients presented the workshop outcome to the plenary session of the ASCAT congress, demonstrating that healthcare professionals are willing to change and listen to the patient voice. Future challenges will be to ensure that research funding is indeed channeled towards projects that address the issues identified by patients.

ACKNOWLEDGEMENTS

This work is generated within the European Reference Network on Rare Hematological Diseases (ERN-EuroBloodNet). FPA 739541

REFERENCES

Exploring the challenge of health research priority setting in partnership: reflections on the methodology used by the James Lind Alliance Pressure Ulcer Priority Setting Partnership, Madden M1, Morley R2, in Res Involv Engagem. 2016 Apr 2;2:12. doi: 10.1186/s40900-016-0026-y.

ANNEX V

ASCAT 2020 DRAFT PROGRAM

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Hematological
Diseases (ERN EuroBloodNet)

Draft
SCD PATIENTS' EDUCATIONAL SESSION'S AGENDA
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 Baba Inusa + Beatrice Gulbis + 1 Patient Representative from ASCAT 2019

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

2. **(5.10 pm – 5.40 pm) Webinar**
   Moderated by Noemi Roy + 1 Patient Representative from EBN representatives or ASCAT 2019 participants

   a. **(5.10 pm – 5.20 pm) Pre-recorded session** with the COVID patients experience from London
   → **3 patients from London, 3 minutes each video** (patients raise questions for the debate in the videos, if any)

      - **Q1** During the pandemic did you avoid going to hospital (during a crisis, for an exam or follow up) for fear of being infected by COVID19?
      - **Q2** Where did you get information during COVID and how did you know if you could trust it? Where them adequate information on how to protect yourself/your child during lockdown?
      - **Q3** Did you continue to receive your care/treatment during lockdown?
      - **Q4** Do you feel that your health situation was different during the COVID-19 period? If yes how?
      - **Q5** What were your support networks during the lockdown?
      - **Q6** Did you find other tools, contact modes for sharing information, ask questions? If yes, which one?

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 EuroBloodNet patients representatives, 3 minutes each video** (patients raise questions for the debate in the videos, if any)

c. **(5.30 pm - 5.40 pm) Live Session Voluntary/Third sector**
Someone from Sickle Cell Society UK or SCDAA, or representative of southern European country to discuss the role of Voluntary/Third sector during the pandemic (10 minutes).
→ **1 patients from UK +1 patient from USA(?) + 1 patient from southern Europe, 3 min each video** (patients giving testimony raise questions during live, if any)

- ○ **Q1** Which was the most challenging aspects?
- ○ **Q2** What were the positives from the experience?
- ○ **Q3** What were the effects of shieling on you? Eg employment/education/mental health/financial

(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 + 1 Patient Representative from EBN representatives or ASCAT 2019 participants

a. **Pre-recorded session** by a patient who would like to share their experience (5 min).
→**2 patients from ASCAT 2019 or EuroBloodNet Patients Representative** (patients giving testimony raise questions during video, if any)

- ○ **Q1** which is in your opinion the added value of having the involvement of a patient in the "peer reviewing process"?
- ○ **Q2** What do you wish you had known before you did your first peer review?
- ○ **Q3** Did you felt that your opinion was taken into consideration during peer review experience?

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 + 1 Patient Representative from EBN representatives or ASCAT 2019 participants

   a. **Pre-recorded session** by a patient who would like to share their experience (5-minute talk each from 2 or 3 patients) - 1 Europe (+1 from another continent? Maybe from Lebanon or Egypt +Sub-Saharan Africa)
   →4 patients from several continents, 3 minutes each video

   - **Q1** What were your reasons for joining the study?
   - **Q2** What were the positives and negatives about joining?
   - **Q3** What messages do you want to give to people who are designing research studies? Which are according to you added values in participating in research?

   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**
   (each experts gives a 5 minutes overview and then answers live questions)
   Moderated by: Raffaella Colombatti + Subarna Chakravorty+ 1 Patient from ASCAT 2019 or EuroBloodNet Patients representatives
   Moderators will address questions to panel

   Panel of physicians: 2 paediatricians, 2 adults haematologist
   (to be identified by Raffaella and Subarna)

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

- ○ New therapies for SCD, **John Brewin, UK** (proposed)

(5.50 pm – 6 pm) ***10 min break***

6. **(6 pm – 6.50 pm) "Meet the patient" 2 parallel sessions with live Q&A**
   Moderated by: Raffaella Colombatti + Subarna Chakravorty+ 1 Patient from ASCAT 2019 or EuroBloodNet Patients representatives
   Moderators will address questions to panel

   1st Parallel session "Meet the patient": Physicians addressing questions to adult patients
   Panel of Patients: 3 patients, patients and parents' patients
   (Questions will be pre-identified)

   2nd Parallel session "Meet the patient": Teenagers patients addressing questions to adult patients
   Panel of Patients: 3 patients, patients and parents' patients
   (Questions will be pre-identified)

(6.50 pm - 7 pm) ***10 min break***

7. **(7 pm – 7.40 pm) 'My life, my word'**
   Moderated by: Mariane de Montalembert + Patient+ 1 Patient from ASCAT 2019 or EuroBloodNet Patients representatives

   → **5 paediatric or young patients, 2 minutes each**

   a. **Pre-recorded session**: 5 paediatric or young patients express their view/questions about their life with SCD.

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

31st of October 2020

1h10 min hours session [Time to be defined]

8. **Q&A session** (Question raised in previously sessions but not enough addressed + new questions and comments) (1h)

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

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