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

European Reference Networks

DELIVERABLE 4.6 ERN-EUROBLOODNET: A MODEL FOR CROSS BORDER REFERRAL SYSTEM

ERN-EuroBloodNet: European Reference Network on Rare Hematological Diseases

EUROPEAN REFERENCE NETWORKS
FOR RARE, LOW PREVALENCE AND COMPLEX DISEASES

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DOCUMENT INFORMATION

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Report document

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

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

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

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Short Description

ERN-EuroBloodnet actions aimed at improving the access to cross-border health from both, healthcare providers and patients perspectives, including the challenges experienced through different situations dealing with cross-border highly specialized procedures for RHD healthcare provision.

Publication Date

30/06/2021

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INTRODUCTION

CROSS-BORDER HEALTH ON RARE DISEASES

Although the vast majority of health care is obtained from providers within the patient's country, this situation may change when highly specialized procedures are required. Given the scarcity and heterogeneous distribution of expertise on certain pathologies and of the allocation of specialized services, it is relatively common for patients suffering from complex disorders that the most appropriate care is offered in another Member State (MS). This situation is commonly faced for the management of Rare Diseases (RDs), defined as those affecting less than 1 person in 2000.

The EU Council Recommendation on an action in the field of rare RDs already outlined these disorders in 2009 out as a unique domain of very high added value of action at Community level due to the limited number of patients and scarcity of relevant knowledge and expertise. This added value can be achieved by gathering national expertise on RDs, which is scattered throughout the MS and organising collaboration between centres of expertise, healthcare providers, laboratories, patients and individual experts within and between MS to offer optimal cross-border services to all EU citizens.

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

HIGHLY SPECIALIZED PROCEDURES IN THE CONTEXT OF RARE HEMATOLOGICAL DISEASES

Highly specialized procedures (HSP) are defined in the context of the network as those procedures that for a number of reasons i.e. economical, lack of expertise or awareness, are not available in all EU-MS, thus preventing the delivery of the best care for EU citizens suffering from a rare hematological disease (RHD) independently of their country of origin.

HSP involve both interventions for diagnosis and for treatment, and their complexity can rely on technological advances or expertise of multidisciplinary team, or both. These HSP are classified as "under the scope" of the Directive 2011/24/EU if they are defined as standards of Care and/or included in the national basket of health services for patients or "out of the scope" in the cases that they are still performed on academic or experimental environment. In these cases, the European cooperation can be produced on the research field.

In this context, ERN-EuroBloodNet identified as urgent needs the identification of the state of the art of the availability of four priority HSPs across EU, as first step for identification of educational needs and implementation of cross-border health actions.

ACCESSIBILITY TO CROSS-BORDER HEALTH BY RARE HEMATOLOGICAL DISEASES PATIENTS

When it comes to RDs patients' rights on Cross-border health, patients and their families could face disinformation and misinformation. This is because of the scarce access to information, lack of available services and lack of existing patients associations that could serve as infopoint. So often patients and caregivers are not aware of RD policy they can benefit from. ERNs could empower patients on navigating health care systems in Europe and raising awareness on cross-border health rights.

In this context, ERN-EuroBloodNet has started playing a key role in Europe for making RHDs patients' burdens and rights visible to scientific community, public-at large and policymakers. ERN-EuroBloodNet could achieve good goals in those domains thanks to the close collaboration with National Contact Points, expert healthcare providers, EURORDIS, ePAGS and European, National or Local patient's representatives groups too.

Accordingly, ERN-EuroBloodNet identified two main areas to play a key role on the assistance to RHD patients to know and use their rights on cross-border health, including the provision of support to RHD patients by the requests received, and the assessment of the accessibility to transfusions and related treatments in a MS of the EU if the patient is abroad.

All in all, the present Deliverable describes the ERN-EuroBloodNet actions aimed at improving the access to Cross-border health from two perspectives:

1. **At the Healthcare provider** level by the identification of availability of HSP across EU-MS as the basis for the identification of needs and further actions ie. educational actions, implementation of cross-border agreements
2. **At the Patients level** by the assistance to RHD patients willing to apply for cross-border health and the gathering of their HSP cross-border accessibility experience

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1. CROSS-BORDER HEALTH ACTIONS TO IMPROVE ACCESS TO HIGHLY SPECIALIZED PROCEDURES

1.1 STATE OF THE ART OF HIGHLY SPECIALIZED PROCEDURES

As mentioned in the Introduction, HSPs are defined as very specific and complex interventions for diagnosis and for treatment, requiring for concrete expertise that due to a number of reasons may not be available in all EU-MS. Accordingly, the identification of the availability of these HSPs at the national level, represents the basis for the identification of gaps to be tackled through cross-border health focused actions.

ERN-EUROBLOODNET MAPPING OF HIGHLY SPECIALIZED PROCEDURES

In this context, two mapping exercises were conducted among **ERN-EuroBloodNet members and non-members addressed to identify the European state of the art of Next generation sequencing (NGS) and Bone marrow transplantation (BMT) on non-oncological disorders** as HSP key for the diagnosis or treatment of many non-oncological RHD and presenting high inequalities for its access among MS. The two questionnaires were conducted among ERN-EuroBloodNet members with the gathering of 50 and 48 and responses received respectively from 12 and 14 different countries. Answers allowed the identification of important gaps among the need and availability of a) NGS for rare anaemia disorders and b) BMT for Sickle cell disease *(Deliverable 3.2 State of the art of Bone marrow transplantation and Next generation sequencing for non-oncological rare haematological diseases in the context of ERN-EuroBloodNet, SGA2017, January 2019).*

This successful approach was expanded to HSPs for **diagnostic procedures of Primary vitreo-retinal lymphomas (PVRL)**, which mapping has been identified of high added value for the achievement of the evidence needed to facilitate the shaping of policies or even the establishment of cross-border pathways as well as setting the basis for the establishment of European guidelines for their diagnosis. As a result 86 answers from 18 countries were compiled, allowing the identification of some key points shedding light on the current situation of PVRL diagnostic panorama on Europe and supporting the need for creating a European guidelines and a task force on the area (*Deliverable 4.4 ERN-EuroBloodNet report on the availability of highly specialized procedures for rare hematological diseases, SGA2019, September 2020).*

The latest mapping exercise conducted has been focused on the **Availability of Transcranial Doppler (TCD) for children with sickle cell disease (SCD),** as a highly specialized procedure required for the adequate management of these patients. In fact TCD is a fundamental part of standard of care (mandatory in all guidelines between 2 and 16 years of age) because it allows to screen and identify children at risk of stroke and in need of a chronic transfusion program for stroke prevention. Specifically, the results of this survey will shed light on the availability of TCD in Europe allowing the identification of issues related to the lack of access, lack of training for staff, lack of adequate protocols for implementation of TCD and treatment afterwards, etc, which could be addressed through dedicated actions in the network. With 82 answers from 17 countries, preliminary results have highlighted a) a better implementation of the TCD guidelines is required, b) collection of educational activities is necessary to teach how to perform TCD to neurologists/radiologists and how to interpret the results to Hematologists and other healthcare staff. c) The creation of a network between centers is crucial for the improvement of TCD implementation, d) Healthcare reports focusing on the need to support healthcare staff are necessary to position TCD as a standard of care for children with SCD in Europe.

ERN-EuroBloodNet mapping exercises on the availability of HSPs provides valuable information for facilitating shaping public health actions and policies addressing disease specific needs in the diagnosis and/or clinical management of the patient at the national level while shedding light into the current EU status of HSPs identified of added value for the establishment of a cross-border referral system.

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1.2 Bilateral Agreement for Bone Marrow Transplant for Sickle Cell Disease pediatric patients between two European Member States: challenges and barriers

Bone Marrow Transplantation is a HSP that is nowadays standard of care for many hematologic conditions both oncological (i.e relapsed or high risk leukemias) and non oncological (i.e hemoglobinopathies). Nevertheless, not all hematology reference centers in Europe have the capacity or the expertise to perform BMT for both oncological and non oncological conditions in the same center or in the same region or country.

Sickle Cell Disease (SCD), an autosomal recessive inherited blood condition, is the most frequent hemoglobinopathy worldwide, but is nevertheless a non malignant rare disease among the countries of the European Union. The clinical picture is characterized by chronic hemolysis, vaso-occlusion and increased susceptibility to infections. Newborn screening in recommended in order to ensure timely beginning of preventive measures (such as penicillin prophylaxis, vaccinations), adequate follow up and implementation of educational strategies to prevent acute severe life threatening complications.

Currently, the only curative option is stem cell transplantation, although gene therapy approaches seem promising for the near future. Stem Cell Transplantation (HSCT) from an HLA matched sibling is considered standard of care for children with SCD and is recommended in many national and international guidelines.

HSCT for SCD should be performed in Bone Marrow Transplant Centers with expertise in managing children with non malignant conditions, since the conditioning regimens, the work up for transplant and the complications can differ from those of children affected by malignant conditions, and require often the advice of the hemoglobinopathy team.

The Irish Health care system had decided that the only HSCT Center of the country, located in Dublin, did not have the capacity to perform HSCT for children affected by non malignant conditions, for whom a partnership with centers abroad was seeked.

Due to a long lasting collaboration in the field of SCD between Ireland and Italy in the framework of the SCATES Project, and due to the participation of both the Dublin Hospital in Ireland and the Azienda Ospedaliera di Padova in Italy to the ERN-EuroBloodNet, a proposal to standardize the referral of patients with SCD and an HLA matched sibling donor from Dublin to Padova for HSCT was developed in 2017.

This report aims to analyze the steps and the challenges encountered in the development of a standardized referral of patients utilizing the cross-border directive for such a complex procedure like HSCT - requiring pre-admission preparation, admission in a Transplant Unit, and post discharge follow up- for a rare non malignant blood disorder. The establishment of the collaboration between Italy and Ireland has shown key hot points for its implementation, including the clinical protocol, administrative burden, legal framework and logistics and specially reimbursement scheme.

Steps in the procedure

- June 2017: decision made by pediatric hematologists to work on this project based on Ireland's need and ongoing cooperation in the field of SCD between Padova and Dublin
- June-December 2017: obtain "in theory" approval by both institutions (AOP in Italy and Crumlin Hospital in Ireland) with letter of Intent from both institutions
- December 2017:
    - beginning of draft of Clinical/Medical Protocol by pediatric hematologists;
    - beginning of Financial review of all previous admissions for sibling HLA matched HSCT of SCD patients by Italian Hospital
- June 2018: Ireland's Team visit to Padova (2 nurses and a pediatric hematologist); final review of Medical and Logistic Protocol
- June-November 2018: review and draft of financial and administrative aspects
- December 2018-February 2019: review and approval of the draft agreement by the Regional Health Authority of the Veneto Region
- March 2019: Draft agreement sent to Irish Hospital in Dublin
- May 2019: positive feedback, but ethical and legal aspects needed to be modified

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CHALLENGES AND BARRIERS FOUND

- HSCT is a complex treatment with an inpatient admission for at least 1 month for the transplant, but before that a month of preparation with several outpatient visits of the patient and the sibling donor; after the discharge at least 4 months of outpatient visits (2-3/week) in the hosting country
- Financial barriers: the lodging is non covered with the E11 module
- The translation for patients speaking a language that is different from the hosting country is not covered
- Different reimbursement procedures for countries
- Different costs in the countries involved

Due to the delay in activating the final steps of the agreement and the insurgence of the COVID 19 Pandemic in 2020 a further discussion was made in Ireland to allow on site HSCT for patients with SCD.

Therefore, although the agreement was not finalized in the end, the discussions and the round tables between experts both in Ireland and in Italy promoted by the ERN-EuroBloodNet's action, served as a driving force to obtain an essential part of treatment also for patients in Ireland where it was absent before.

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2. CROSS-BORDER HEALTH ASSISTANCE TO PATIENTS

2.1 ERN-EUROBLOODNET CROSS-BORDER HEALTH ASSISTANCE TO RARE HEMATOLOGICAL DISEASES PATIENTS: AN INFOPOINT FOR PATIENTS DEALING WITH CROSS-BORDER HEALTH RIGHTS

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.

In addition, ERN-EuroBloodNet aimed at providing cross-border health assistance to RHD Patients, as ERN are organized for sharing information and coordinate of health care across the European Union. The network could share information on Cross-border health rights and play the role of moderator among cross-border health actors: National Contact Point, Healthcare providers, health authorities and people dealing with the procedures.

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.

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

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

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

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

ERN-EUROBLOODNET INFOPOINT EXPERIENCE

Since the launching of the ERNs, the ERN-EuroBloodNet has received 16 requests of cross-border health assistance, especially focussed on assisting patients navigating the healthcare social systems in Europe by offering a mediation among National Contact Points for Cross-Border Care and hospital administration. In details:

1. 2018. Betha Thalassemia patient moving residence from Italy to France and wanted to access to reimbursed transfusion. Not a reason of cross-border health assistance. Obstacles: passing from one social security to another require time in which the patient had complications in accessing transfusion. Solution: SCD patients fount a job and automatically obtained the social security assistance.

2. 2019: a Betha Thalassemia patients moving from Italy to France for a short-term job contract. Patient wanted to access to reimbursed transfusions. Not a reason for cross-border health assistance. Obstacle: passing from one national social security assistance to another require time in which the patient has complications in accessing healthcare services. Solution: the SCD patients helped with local administration.

3. 2019. Successful example of cross-border health Assistance for Chronic conditions and programmed cares. Italian student in Erasmus study program in France had to schedule cares for thalassemia. ERN contacted the French NCP for cross-border health and asked mediation among patient' home and host countries social security funds. Patient benefit from the temporary cross-border mobility.

4. 2019. SCD patient (20 years, France) studying in Madrid had burdens in receiving transfusion for lack of knowledge. ERN gave the availability to contact expert physicians in Spain but the patient did not answered back.

5. 2019. Request for finding expert centers in Scotland that could follow the patients' management of three SCD pediatric patients moving from France. Not cross-border health Case. But the ERN identified HCPs and physicians in Scotland.

6. 2019. Request for finding SCD expert center in Spain for an adult patient moving there. Not cross-border health Case. But the ERN identified expert center.

7. 2020. Request from an Italian Thalassemia patient for finding an expert center in UK. Request stopped with the outbreak of the covid 19 pandemic.

8. 2020. PNH Adult Patient from Bosnia (not EU MS) needed to access to Clinical Trial. ERN support has been provided by PNH expert member offering to edit an official request for access to treatment in patient own country (Bosnia).

9. Three different cases have been registered respectively one in 2019 and two in 2021 for accessing a cost-effective treatment for PNH in their own country: Belgium, Lithuania and Poland. These requests have been solicited by ERN-EuroBloodNet ePAGs for Aplastic Anemia and PNH. The action has required a coordinated plan among European Network of Patients Association lead by an ePAGs, PNH experts members of the Network and ERN-EuroBloodNet coordination team. For the case of Belgium and of Lithuania, ERN-EuroBloodNet PNH experts stated in official clinical document that that treatment is crucial for patient life, after receiving clinical records. For Poland the request was not linked to one specific patient, so EBN PNH experts and PNH ePAG prepared letter to Health Authorities recommended the access to the treatment as scientific evidence of its efficacy. For Belgium case, the patient got the access to treatment. For Lithuania the reimbursement of the treatment has been accepted by health authority, the patient is waiting for taking the treatment. For Poland the access to treatment has been denied by health authority.

10. 2021 for an Italian adult patient affected by Anemia Fanconi asking for an expert consultation of Bone marrow transplant side effects in France where he received a transplant of bone marrow when he was a child. Patient will be accompanied in Cross-border Health related procedure for obtaining the reimbursement of the expert visit. The case is ongoing.

11. 2021 and still ongoing, for a second opinion on a case of Composite Lymphoma.

12. 2021 for a SCD adult patient participating in an ERASMUS project in Germany. Request was arrived via the French National Network of Rare Blood Cell Disorders. The ERN fount the expert and inform the patient that cares are in the in light of European legislation (Regulations (EC) 883/2004 and 987/2009), patient is considered to be temporarily residing in Germany during his/her studies.

13. 2021 Two cases for SCD adult patients searching for clinical expertise abroad because of moving in another country.

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

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

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

Considering the important number of assistance' requests received by patients for accessing transfusions in a European Member States, 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.

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.
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

A test was conducted on three voluntaries patients for reshaping the final version. Survey has been translated into Italian, French, and Spanish and conducted 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.

SURVEY RESULTS

1. A SCD Patient, (35 years old, France) moved in Netherlands for professional reasons. Patient did a vaso-occlusive crisis and needed a transfusion. Hospital did not perform it because of a lack of knowledge of the disease.

2. A Betha Thalassemia patient (36 years old, Italy) transferring residence to France and wanted to access to reimbursed transfusion 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.

3. PNH patient (54 years old) wanted to go abroad but no transfusion were available. Answer n/a. Patient declared no obstacle was found in requesting transfusion.

4. CDA patient (31 years old), did not experience accessing transfusions abroad.

5. A Betha Thalassemia Mjor patient (43 years old, Italy) wanted to go to USA for a clinical trial did not find any obstacles in receiving transfusions.

6. SCD patient (20 years, France) studying abroad, in Madrid found difficulties in receiving transfusion for lack of knowledge of the disease.

As a result, 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 obstacles it results very important to integrate ERNs at National Level

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CONCLUSIONS

One of the most ambitious objectives of the ERN-EuroBloodNet is the promotion of accessibility and patients' awareness of Cross-border Health in the field of the RHDs, especially for those HPSs which, despite of their major importance for the best healthcare provision to patients, may not be available in all EU-MS. In this context, the identification of the availability of key HSPs at the EU level through ERN-EuroBloodNet mapping exercises represents a solid the basis for the identification of gaps while allowing the implementation of corrective measures, either based on educational or cross-border health focused actions to achieve the best health care provision to RHD patients.

On the other hand, the pilot project for the establishment of a cross-border health agreement among 2 ERN-EuroBloodNet Helthcare providers in Ireland and Italy for bone marrow transplantation in children with SCD has provided useful lessons on the steps to be taken for the procedure while allowed the identification of key challenges to be considered in the future. Although the final steps of the process were not finalized and the arrival of COVID pandemic forced the on site HSCT in Ireland without the need of the cross-border agreement, the discussions between experts promoted by the ERN-EuroBloodNet's action, served as a driving force to obtain an essential part of treatment also for patients in Ireland where it was absent before.

Regarding the promotion of Cross-border health awareness among patients, it is essential the collaboration with National Contact Points, 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. From ERN-EuroBloodNet experience with the Infopoint for patients dealing with Cross-border health, it has emerged the need of integrating the ERN at national level for better dealing with Cross-border health Policy at health authority level (as social securities management) and make them recognized at local level by hospital administration. Finally, it has made it visible the necessity of raising awareness on Cross-border health Rights among patients and caregivers community. Potential future actions identified to improve this awareness are a) Programs shaped with European Network of Patients Associations for disseminate knowledge on cross-border health policy at local level, b) Make the assistance visible during conferences, webinars, educational trainings, etc. c) Developing some media plan on cross-border health rights for patients living with rare conditions.

Furthermore, the results from the survey on accessibility to transfusions and related treatments, has shown major obstacles in accessing these type of HSPs. 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.

In conclusion, ERN-EuroBloodNet Cross-border health strategy has allowed not only to directly assisting patients and their families in dealing with access to information, accompaniments to procedures and moderations among health authorities and local administration, but also to have a first analysis of obstacles that patients deal when it comes the situation of: searching for expertise abroad, moving abroad, access to a health service abroad or access to a cost-effective treatment or clinical trials in an European Member State. Considering those fruitful results and outcomes obtained from the initiatives, same actions will continue in the running coordination of the ERN-EuroBloodNet. In addition a new strategy for strengthen the patients awareness on cross-border health rights will be proposed for integrate an exhaustive strategy of patients' empowerment and patients' assistance.

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