

SUPPORTING THE ENGAGEMENT IN ERNs OF UNDER-REPRESENTED PATIENT COMMUNITIES EUROBLOODNET

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Activity	Supporting the Engagement in ERNs of under-represented patient communities
Area	<i>Communication and dissemination / Education and training/ Governance</i>
Duration	2019
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1. SUMMARY

i Sickle-Cell Disease (SCD) is due to a genetic defect in Red Blood Cells (RBCs) production that induces the sickle-shape of RBCs with a reduced amount of hemoglobin, a protein addicted to carry oxygen throughout the body. Patients suffering from this condition have severe painful crisis, increased risk of strokes and infections and sudden acute chest syndromes that impact hugely on everyday life. It's therefore a life-threatening condition. In the context of ERN-EuroBloodNet within the hub for non-malignant hematological diseases, Sickle-Cell Disease is the only condition without an ePAG advocate as SCD patients' organizations are scattered and not coordinated across European countries. This lack of SCD patient representatives means that their unmet needs cannot be taken into consideration to inform ERN activities. Therefore, EuroBloodNet ERN has decided to support this patient community to cross-collaborate so that they can identify their common needs, strategic priorities and representatives to allow them to be meaningfully involved in the ERN. The establishment of an alliance or providing a space for discussion, although informally, can contribute a lot in including sickle-cell patients' needs in the framework of EuroBloodNet activities, improving the care and increasing the awareness of the European scientific community about these patients and their needs. The added value of this experience is to showcase how to support a given patient community to get organised across Europe so that their voice can be heard in ERN operational and strategic discussions. It may constitute a model for other rare or complex diseases where work at European level is currently underdeveloped or inexistent.

2. INITIAL SITUATION

i From the establishment of ERN-EuroBloodNet, patients' involvement has been widely accepted and welcome by the Network's Coordinator. The ePAGs group was actively involved in developing the initial application of the European

Call for ERNs and patient advocates were included in the Network's governance. The patient community for rare hematological diseases has a very distinct background and a long story of patient advocacy both for malignant and non-malignant diseases. The groups of patients' representatives belonging to these two main areas have developed since many years a sort of community for facing transversal needs and are therefore linked and used to work together.

However, the ERN lacks a patient advocate representing Sickle-Cell Disease because patient organisations have traditionally worked only at national or local levels and have no previous experience of networking and collaborating at European level. This directly translates into an under-representation of their needs in the ERN discussions and activities.

Sickle-Cell Disease is a non-malignant condition affecting mainly black African and middle-east populations. Most of the patients in Europe come originally from developing countries, where this condition is endemic; they are living therefore many problems of inclusion for different reasons. Some of these patients have established their own organizations for advocacy, but the wider patient community appears quite unaware of the existence of such organizations and living a quite varied life experience mainly due to the socioeconomic context where they live. These organizations were mainly set up to address specific needs of their community of reference, but they lack a holistic vision and the experience of networking that is pivotal for being successful.

3. CONTRIBUTION TO PATIENT ENGAGEMENT AND/OR IMPROVEMENT OF CARE

i The project aims to provide a space for discussion for this patient community in order to support them in organizing their work at European level and facilitating their engagement in EuroBloodNet's activities.

The support will be delivered specifically by the following actions:

- Organizing different meetings, grouping the patients and their caregivers having the same language (Italian, English and French) in different locations in order to explain to the existing organizations' representatives the rationale of EuroBloodNet's activity, listening to their comments and the problems they are facing to access health and social care. The facilitators for these meetings will include one ePAG for hemoglobinopathies, one clinician with specific experience in this field and one person of the EuroBloodNet management team. The facilitators' role is to drive the discussion ensuring that all participants understand and are brought up to speed with the activities of ERN-EuroBloodNet and that all the patients' needs are accurately reported.
- Proposing a training plan for the patients' representatives that they will select.

The final aim is to organise a plenary meeting at European level, maybe in the context of an EuroBloodNet Board of Network meeting, where the sickle-cell patients can appoint officially a member to represent them in EuroBloodNet ePAG. Through this action also the unmet needs of these patients will take into consideration and contribute to shape the activities for the upcoming years.

4. SUCCESS FACTORS

i At the moment this project is still ongoing, therefore it is early to present specific achievements, but the following aspects have been critical to drive forward the project:

- **Having a well-established and mature patient community** for hematological diseases, helped to identify the need of an under-represented patient community early on, when the ERN was launched, and come up with an action plan to help these patients.
- **Partnering with clinicians in the early phase of the project to gather information on the current situation and get their support.** One of the first objectives was to get the facts and understand what was

the situation of these patients at European level. To do this we partnered with the clinicians, who are the ones having direct contact and access to these patients, and brought them on board in this early phase.

- **The initiative got traction because it builds on a public health challenge** identified by the scientific community. The number of SCD patients has increased significantly in Europe in the last years and clinicians need to address this challenge and understand how to best treat this condition.
- **Involving the ERN management team** in this exercise and as facilitators in the meetings is essential to have a champion in the ERN, who is not a patient advocate, and ensure the continuous support of the ERN Coordinator.

5. LESSONS LEARNED



- **We need to find patient advocates that are able to represent the real needs of the diseases covered by the ERN.** When approaching a health problem, even if a given condition can be similar to others or share similar problems or challenges, such as Sickle-Cell Disease and thalassemia, it is preferable to involve the people that are living with that particular condition to represent and explain their needs, rather than to make wrong assumptions.
- **Networking at European level takes time and needs to follow a step-wise approach. Each patient community needs to find their own way and we should avoid imposing a model.** The lack of a well-structured patient organization at European level, suggested the need to first create a space for discussion where all the stakeholders could express their views, share comments and expectations, rather than establishing from the outset a legal organization and impose this as a solution to the patients' representatives. It will be up to the SCD patients themselves to decide when and if they would like to move from an informal alliance to the establishment of a legal entity such as a European organization for patients with sickle-cell disease.
- **Partnering with clinicians has been critical to move things forward.** This experience has shown how the combined efforts of ePAG patient advocates and clinicians can effectively support the development of an informal European alliance or network of patients for an under-represented rare disease, and eventually facilitate the emergence of ePAG patient advocates that will eventually be able to represent that disease in the Network.
- **A good understanding of the policy context helps to identify opportunities and build partnerships to improve patient engagement in the ERNs.** The management of SCD patients is currently a public health priority for the scientific community. We understood this, and proposed an action that would help the clinicians to address this challenge more effectively.

6. UPDATE NOVEMBER 2023

Link to article on the Orphanet Journal of Rare Diseases:

- **Sickle cell disease: embedding patient participation into an international conference can transform the role of lived experience**



