



European Haemophilia Consortium

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EHC Round Table of Stakeholders

National Haemophilia Council: From Concept to Reality

Meeting Report

European Parliament, Brussels, 1st December 2014



Executive Summary

On 1st December 2014, the European Haemophilia Consortium (EHC) organised its third and final Round Table of Stakeholders of the year at the European Parliament in Brussels, Belgium. The topic of the Round Table was 'National Haemophilia Councils: From concept to reality' and it explored the value of patient engagement in the organisation of haemophilia care. Over 50 participants representing the EHC membership, physicians and industry joined the meeting.

Member of the European Parliament (MEP) Mrs Nessa Childers opened the event and welcomed participants to the European Parliament. She said she was pleased to see civil society actively engaged in the organisation of healthcare and noted that with recent financial pressures any initiative that can increase the cost-effectiveness of health systems is welcome. Dr Cristian Busoi MEP co-chair of the event welcomed participants through a video message.

The event continued with presentations defining the concept of National Haemophilia Councils (NHC) and their benefits both from a patient's and physician's perspective. Benefits include: cost-effectiveness with the example of Ireland where the consumption of coagulation factor concentrate has doubled in 12 years with no increase in the budget. It also showed that the centralisation of the planning and organisation of healthcare can facilitate the implementation of additional services that can improve both cost-effectiveness and patients' treatment. For example, NHC facilitate the development of patient registries, electronic patients' records, electronic tracking systems for clotting factor stocks, to name a few. Central management of the haemophilia care budget also improves the allocation of resources according to centres' needs and facilitates the implementation of a review system where all services are evaluated on the same basis. Finally, patients are considered as partners and they are able to provide feedback on healthcare services from the user's perspective.

The event also featured three presentations from Poland, Romania and the United Kingdom (UK) where the organisation of haemophilia care varies greatly. These experiences underscored the benefits of NHC. In fact, in Romania where a NHC was established only in 2013, the level of treatment and the budget allocated to haemophilia care has considerably improved with the budget for Haemophilia increasing by 80%. Patients are now part of the official process and have a real voice in the development of haemophilia care. In Poland, the lack of official patient involvement in the organisation of haemophilia care has resulted in unexplained budget cuts. On the other hand in the UK the fragmentation of healthcare services makes it difficult for people with bleeding disorders to access comprehensive care services. The latter two examples show the clear dissatisfaction of healthcare systems' end-users and this could be improved if patients were listened to and integrated in to the decision-making impacting their lives.

Finally, Camille Bullo from the European Patients Forum (EPF) presented on the concepts of patient involvement and patient empowerment. She explained that European legislation and soft laws were increasingly inclusive of these concepts but that however they are still not fully implemented in Member States. She also warned that both concepts needed to be applied in a meaningful way and not just to tick boxes.

The event concluded with discussions and the following recommendations were developed:

- European countries should acknowledge that patients are the end-users of healthcare services and that as such they have considerable knowledge and expertise about the functioning of healthcare services;
- European countries should acknowledge that patient involvement is essential to develop healthcare services that provide efficient services to its end-users: patients;
- European countries need to treat patients as partners, listen to them and take into consideration their opinions as well as those provided by healthcare professionals and others involved in the healthcare system;

- European countries should acknowledge that haemophilia, von Willebrand Disease and rare bleeding disorders are complex conditions that impact all aspects of life of those living with them. These bleeding disorders have a direct impact on all other health issues encountered by people with these conditions and this results in the need for all healthcare professionals treating these patients to be informed about their condition and how best to treat them. This is why any healthcare professional treating a patient with bleeding disorders must be able to communicate with other healthcare professionals treating the patient and to be able to access resources on how the treatment he or she will prescribe will impact other aspects of the patient's disease;
- In order to better utilise resources, to improve cost-effectiveness and to improve treatment for patients with bleeding disorders, each European country should establish a National Haemophilia Council (NHC) to oversee the organisation and delivery of health services for haemophilia and other bleeding disorders. This body shall recognize that bleeding disorders are complex conditions needing a multi-disciplinary approach;
- NHC should be composed of haemophilia clinicians or representatives of the haemophilia comprehensive care team, government agencies, payers and patients. All participants shall be considered as equal partners and formal participants;
- NHC should advise on matters related to the allocation of budget and the organisation of haemophilia care. The NHC should look at how services can be improved for both providers and end-users within the budget set by the national government;
- NHC shall support initiatives that will improve quality of care and foster research, such as the establishment of national patient registries, electronic patient records, medicinal products stock management systems, protocols and guidelines for the treatment of patients with bleeding disorders, audits of haemophilia comprehensive care centres and certification of haemophilia treatment centres based on the EUHANET¹ guidelines;
- Patients involved in NHC should be selected by the national haemophilia patient organisation. They should receive proper training and support from their organisation to participate in the activities of the NHC.
- The establishment in each European country of an NHC is in accordance with the recommendation from the Kreuth III initiative² (***Recommendation 1: To optimise the organisation of haemophilia care nationally, it is recommended that a formal body be established in each country to include the relevant clinicians, national haemophilia patient organisation, health ministry, paying authority and (if appropriate) regulatory authorities.***)

Speakers included:

- Ms Nessa Childers, Member of the European Parliament
- Mr Brian O'Mahony, President, European Haemophilia Consortium
- Dr Barry White, Irish National Haemophilia Centre – St James Hospital, Dublin
- Mr Radoslaw Kaczmarek, EHC Steering Committee Member
- Mrs Liz Carroll, CEO of the UK National Member Organisation (NMO)
- Mr Daniel Andrei, President of the Romanian NMO
- Ms Camille Bulot, European Patients Forum

¹ EUHANET is a project aimed at establishing a network of haemophilia centres to work together on a number of related projects to improve the care of European citizens with inherited bleeding disorders.

² Giangrande P. et al., Kreuth III: *European consensus proposals for treatment of haemophilia with coagulation factor concentrates*. Haemophilia 20 (2014): 322-325.

Introduction

The event was opened by Mrs Nessa Childers MEP (Ireland/ S&D), who explained that the topic of access to medicines and healthcare services has been recently widely discussed in the European Parliament.

Mrs Childers stated that she was pleased to chair an event showing that the deep involvement of civil society in the organisation of healthcare can lead to cost-effective access to medicines. She stressed that, although healthcare is a competence of Member States, the European Parliament has a say in terms of non-discrimination and access to healthcare services and medicines, which is a fundamental right in Europe.

She also noted that the European Parliament worked on legislations such as the Transparency Directive and the Cross-Border Healthcare Directive, which encourage participation from the civil society in the organisation of healthcare. The European Parliament also votes on the budget of the European Commission, which includes the EU Health Programmes that finance projects including research, awareness-raising, post-marketing surveillance and pharmacovigilance. These programmes also promote best practices and cooperation amongst Member States to improve healthcare services and access for people with rare diseases.

Finally, she introduced the Round Table speakers and the discussion topics.

Dr Cristian Busoi MEP (Romania/ EPP) co-hosted the event and welcomed participants to the event by video message.

National Haemophilia Councils

Mr Brian O'Mahony, President of the EHC, started his presentation by noting that it was World AIDS Day. He commemorated the haemophilia community that had been affected by HIV due to the contamination of their treatment.

He then proceeded to present in more detail the concept of NHC, a formal or informal body where various stakeholders with a competence and interest in the access to haemophilia care are convened to plan its organisation and delivery. Mr O'Mahony reminded that the establishment of a NHC was one of the seven recommendations of the Kreuth III initiative³ and it is therefore relevant for all EHC members both in the European Union (EU) and outside the EU. Currently only Ireland, Georgia and Romania have a formal NHC.

In Ireland, for instance, the NHC was formed in 2004 and it has a statutory basis, which in Mr O'Mahony's opinion is important as it allows patients to be formally involved and to have an official role in decision-making with regard to the organisation of haemophilia care. Amongst the NHC competencies are, for example, the allocation of haemophilia budgets, the development of national guidelines for the treatment of haemophilia and the organisation of audits for the haemophilia treatment centres.

The value of a NHC lies in the ability for a single body to coordinate the organisation of healthcare for haemophilia nationally by bringing together all key stakeholders to agree on the priorities for the patient and medical communities. It also allocates resources according to a set national strategy developed according to patients' and treatment centres' needs. Furthermore, NHC meetings provide face-to-face time between healthcare providers, Ministry of health officials and consumers and this enhances dialogue and better understanding of each other's needs, which facilitates the medium to long-term planning of healthcare services.

Another advantage of establishing a NHC is to bypass local politics and tensions. This facilitates collaboration to implement a number of projects, which are more easily run through a centralised system,

³ The Kreuth III initiative is a meeting held in 2013 organised by the University of Munich, the Rudolf Marx Foundation and the European Directorate for the Quality of Medicines and Healthcare (EDQM) on the optimum use of clotting factors and immunoglobulins. It resulted in seven recommendations for the optimal treatment of haemophilia care. Further information can be found here: <http://www.ehc.eu/events/whd-2014/>

such as patient registries, national procurement for medicinal products, electronic patients' records and medicinal product stock management.

A centralised approach to haemophilia care will also make the access to high-cost treatment more egalitarian. Normally, hospital services with higher expenditures tend to come under the radar of the hospital management for their high spending and are penalised or restricted in their spending. If this high-cost services come under a centralised haemophilia budget and are separated from the hospital budget then the service staff will be able to work according to good clinical practices and not to budget containment policies. Furthermore, this system will ensure a fairer distribution of national haemophilia budget according to the centre's needs.

Finally, Mr O'Mahony noted that the NHC also contributes to a higher product use and better budget management. For example, in 2004 Ireland used 3.7 International Units (IU) per capita of FVIII while in 2014 it used 8.1 IU per capita even though the budget did not increase since 2004, which clearly demonstrates cost-effectiveness and the benefits of a co-ordinated national approach.

Physician's perspective

The event continued with a presentation from Dr Barry White, Director of the Irish National Haemophilia Centre. Dr White outlined the current state of haemophilia care in Ireland. A country of 4.5 million people, Ireland has currently 545 people with haemophilia, 555 people with von Willebrands Disease and 285 people with rare bleeding disorders. People with bleeding disorders in Ireland have access to home treatment and prophylaxis treatment for all children and adults. The country uses recombinant clotting factor and haemophilia care in Ireland is provided by three comprehensive care centres.

Dr White then proceeded to explain why there was a need for an NHC. He stated that currently healthcare systems are facing major challenges in the areas of safety, cost and resource management, experience and compassion, and patient self-care. He furthermore explained that today's healthcare systems should aim to improve their performance, and in his opinion, no one is better placed to provide feedback on healthcare services than patients themselves. Patients' organisations need to evolve from their role of advocates to partners. In order to face challenges and improve performance, healthcare systems should have in place governance systems with a supporting infrastructure. Standards need to be in place as well as clear performance indicators that are driven by users and providers. Finally, each healthcare system needs to have both risk management and quality improvement systems in place. All of these parameters are key to avoiding healthcare systems failures such as, for example, the contamination of people with haemophilia (PWH) with HIV and Hepatitis C (HCV) in the 1980s and early 1990s.

The Irish NHC was established in 2004 and gathers patients, clinicians, payers and representatives from the Irish Department of Health. The NHC also works closely with the Irish Haemophilia Product Selection and Monitoring Advisory Board, responsible so far for the organisation of national tenders on the basis of safety, efficacy, quality and cost. The NHC also oversees external audits for haemophilia centres every three years, which has resulted to significant improvements of services in the past ten years - for example, facilities for patients with reduced mobility, the reduction of coagulation factor waste, improved cost control and the management of adverse events.

To conclude, the key achievement of the NHC, in his opinion, is the institutionalising of partnership with patients.



Dr Barry White presenting during the Round Table

National Haemophilia Councils: countries' perspectives

Representatives from Poland, Romania and the UK gave an overview of the organisation of haemophilia care in their countries. They showed that there is a very diverse approach to the organisation and delivery of healthcare services.

Of the three countries, only Romania has a formal NHC, while the UK is faced with a fragmentation of its healthcare services, which means that at the moment a centralised approach to haemophilia care is almost impossible. In Poland, on the other hand, patients have been faced with random and non-transparent cost-cuts, which could not be prevented as patients are not formally involved in the organisation of haemophilia care.

The Polish experience

Radoslaw Kaczmarek, member of the EHC Steering Committee and member of the Polish National Member Organisation (NMO), explained that patient involvement can help to develop a more patient-centred healthcare service that fulfils the needs of patients, as demonstrated by the 2012 EHC Survey on the State of Haemophilia Care in 35 countries. The survey showed that countries with higher patient involvement enjoyed better access to care. However, in many countries, patient organisations are still not perceived as true partners, which in his opinion is a wasted opportunity.

Poland is a country with a population of 38 million people and over 4,000 people suffering from bleeding disorders. A massive change in the access to care for people with bleeding disorders was noted following the fall of the Union of the Soviet Socialist Republics (USSR) with not only an increase in treatment regimens but also the establishment of a patient organisation and the ability to advocate with the government. In 2005 the Polish government established a first national haemophilia programme and in 2008 all children received primary prophylaxis. In 2012 a second programme started with the objective to provide prophylaxis in adults and improve comprehensive care. Unfortunately, in 2014 this programme's budget was cut in half with no real explanation and patient participation was described as *unnecessary and redundant*.

Mr Kaczmarek concluded his presentation by explaining that, had there been patients sitting at the table with decision-makers, this situation might have been avoided. Also, he explained how patients' perspectives can support Health Technology Assessments (HTAs) by providing information about quality of life instead of basing decisions on clinical outcomes only.

The UK conundrum

Liz Carroll, CEO of the UK NMO, gave a brief overview of the history of the haemophilia Alliance. Formed in 1999, the Alliance brought together patients and healthcare professionals to promote best practices and innovation through advocacy to government, politicians and the public. The objectives of the Alliance covered several areas including the development and dissemination of guidelines, HTAs, advocacy, audit of haemophilia centres and support of research.

Unfortunately, as of 2013, healthcare is a competence of the four British nations: England, Wales, Scotland and Northern Ireland instead of the Department of Health. Furthermore, organisation and commissioning of healthcare services in each nation is done by specialty, which is particularly counter-productive for comprehensive care as each medical specialty that is part of the comprehensive care for haemophilia will be looked at and funded separately. This does not allow for co-operation amongst various healthcare professionals and also means that patients' organisation have to deal with each medical area separately knowing that the people in charge of the organisation of these services will only have a narrow perspective on the service they are responsible for. Also, these entities do not see the benefit of the Alliance.

This situation could be improved with the establishment of a NHC that would bring to the table all those responsible for haemophilia care. The Haemophilia Society will now seek to achieve that in order to improve haemophilia care.

Success in Romania

Daniel Andrei, President of the Romanian NMO, presented the situation in his country. Romania is a country with many disparities in terms of level of haemophilia care and is unfortunately one of the countries in Europe with the lowest treatment rate for bleeding disorders. During the 2013 Annual Conference of the EHC, a Memorandum of Understanding was signed between the Minister of Health, the Romanian NMO and the EHC, which established a NHC with full participation from the patients' representatives. Since then the NHC has been functional and during a press conference held on World Haemophilia Day in 2014, the Ministry of Health announced an 80% increase in the budget for haemophilia care. This is clearly a success for the Romanian NMO, which intends to continue the good work.

Putting Patients at the Centre of Healthcare

Ms Camille Bulot, Membership Officer of EPF, provided a presentation on the patient's involvement in healthcare decision-making. EPF is an umbrella organisation representing over 60 patient's groups with the objective to *ensure that the patient community drives health policies and programmes that are adapted for patients.*

She started her presentation by stating that patient's involvement is both a right, as stated by the Alma Ata Declaration⁴ from the World Health Organisation, and an operating principle⁵ of European health systems. She noted that healthcare is a changing landscape with constant innovation, which can be transformative for patients' lives. However, there is also constant financial pressure to seek valuable innovation that can truly impact patients' quality of life. In order for this innovation to be relevant to the end-users, it is increasingly important that patients are involved in the whole development process of innovation. She noted that in theory health systems are moving towards a system where patients have become active actors both in their own medical decisions and at a higher health-policy level. However, despite an increasingly number of active patients' groups, this is not always the case in practice. She described two concepts that are widely used in European health policy but often not properly implemented: patient empowerment and patient involvement.

Patient empowerment is a process by which patients can gain control of their lives and are able to make informed decisions regarding their health and medical situation; furthermore they are able to express their needs and devise strategies for involvement in decision-making. On the other hand, patient

⁴ "The people have the right and duty to participate individually and collectively in the planning and implementation of their healthcare." Alma Ata Declaration – Principle IV (1978, WHO).

⁵ "All EU health systems ... aim to involve patients in their treatment, to be transparent with them, and to offer them choices where this is possible... to offer individuals information about their health status, and the right to be fully informed about the treatment being offered to them, and to consent to such treatment. All systems should also be publicly accountable and ensure good governance and transparency." Council Conclusions on common values and principles in European Union Health Systems, 2006.

involvement is when patients, thanks to their knowledge and relevant experience, take an active role in decision-making that will result in concrete actions for the patient community. The involvement is planned, appropriately resourced, carried out and evaluated. The definition of patient involvement fits into the role of patients on NHC. Ms Bulot warned that patient involvement should be meaningful and patients should be considered as equal partners; failure to do so, will equal no patient involvement.

At the EU policy level, patient-centeredness is recognised as a key dimension of quality and the concept of patient empowerment can be found in parts of EU legislations⁶ and soft laws. The European Medicines Agency is perhaps the most advanced in terms of patient involvement with patients providing insights on many initiatives from committee work to the review of medical literature to providing direct evidence of benefits for treatments. Patients are also increasingly demanding to be involved in HTAs and in the work of HTA agencies.

Ms Bulot concluded her presentation noting that patients, in order to complete meaningful work, needed to abide by the same standards of transparency and work ethics as all other partners. This includes, disclosing any relation with industry and ensuring good governance.

Discussions and Conclusions

Following this last presentation, the floor was opened for questions and discussions.

Representatives from Estonia confirmed what had been mentioned in the presentations about resistance to change and that often patients' perspectives were not taken into consideration. In order to change this, patients' groups need to align with other stakeholders or find a champion amongst other stakeholder groups that will support their involvement in decision-making.

They also stated that support from a European organisation may be more effective and beneficial as it gets away from national politics and because it shows a certain consensus and support from patients across Europe. It was also noted that patients' groups' actions were most effective during election years. This comment was supported by Mrs Childers, who noted that politicians need to hear from patients and other representatives from civil societies as they are there to represent them.

Mr O'Mahony underscored the importance of data collection. Politicians needs to be provided with successful examples that are implementable and feasible, backed by hard data.

A representative from the Netherlands noted that it is not always easy to formalise NHC and that as long as these bodies are informal, there is no guarantee that their input will be taken into consideration and influence health policy. This is why it is so important to formalise NHC with terms of reference and to ensure that the right people are in the room. Patients in the room need to be properly selected and trained so that they can make a valuable contribution and are not merely present so that authorities can tick a box on patient involvement. Patient's groups should be organised and have a clear objective and speak with one voice. It is very important to build relationships with the media. This will ensure the meaningfulness of the representation. Furthermore, it was stressed that the pharmaceutical industry should not be on NHC because there is a clear conflict of interest.

Participants suggested that the EHC should develop some advocacy material on how to set up a NHC.

⁶ Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare and Council Directive 93/42/EEC of 14 June 1993 concerning medical devices.