



FINDING WAYS FOR BETTER INCLUSION OF PEOPLE WITH INHIBITORS IN THE LIFE OF NMO

4 October 2018, Brussels, Belgium

REPORT





BACKGROUND

The EHC Pre-Conference Workshop “Finding ways for better inclusion of People With Inhibitors in the life of NMO” took place on 4 October 2018, prior to the EHC Conference in Brussels. It brought together more than 30 NMO representatives, both people with inhibitors and NMO leadership, as well as the EHC Inhibitor Working Group members and observers.

The workshop aimed to explore the

challenges with ensuring participation of people with inhibitors (PWI) in the life of the EHC National Member Organisations and seek to **develop tailored action plans for inclusion** and involvement of PWI in their NMOs.

Through a variety of interactive methods, the programme of the workshop focused on the diverse aspects of addressing the needs of people with inhibitors in the EHC NMOs.



WELCOME AND INTRODUCTION TO EIN

The workshop started with a short introduction to the European Haemophilia Consortium (EHC) and the European Inhibitor Network (EIN) programme.

The European Inhibitor Network is a programme of the EHC, which seeks to address the variety of needs of people who have haemophilia with inhibitors. The number of people with inhibitors is often very small nationally. Therefore, it can be difficult to meet the specific needs of this sub-group of the haemophilia community and address the personal and systemic challenges faced.

Those challenges can include lack of information, insufficient treatment, mobility

constraints, social and financial barriers, psychosocial impact, etc. This situation can be changed by acting on a European level – just as the European Inhibitor Network seeks to do!

The programme aims to: **understand the needs** of people with inhibitors and look at ways to meet those needs; **build a community** of people with inhibitors and their families and caregivers; allow mutual support, education and empowerment through online and face-to-face exchanges. It also seeks to provide people with inhibitors with the information, education and advocacy training to **engage directly with decision-makers**.

Finally, it is the objective of the EHC to work

closely with medical experts **towards a framework** for treatment and care that could possibly bridge the gaps of inhibitor treatment in Europe and ensure that every person in Europe who has haemophilia with inhibitors can receive appropriate treatment and care.

In order to break the ice and get to know each other better the participants were led into an ice-breaker during which they had to form groups based on different criteria that they shared, for example the colour of their eyes, the number of siblings they had or their preferred genre of film.

NEEDS ANALYSIS:

PERSONAL AND ORGANISATIONAL PERSPECTIVES

The aim of this session was to understand: 1) the specific needs of each NMO that took part in the workshop, in order to better include PWI in their work, and 2) the needs of PWI in the respective countries that need to be met through the work of the NMO.



The session started with a patient perspective outlined by **Mr Mirko Jokic** (EHC Inhibitor Working Group, Serbia). Mirko shared his story of being a person with haemophilia and inhibitors, starting from growing up as a child with severe haemophilia to developing inhibitors, finding his way to his NMO and getting actively involved in its work.

The consistent message through Mirko's presentation was that each patient with inhibitors has **different needs at different life stages**. A child (and his parents) with inhibitors who is missing out from school due to frequent bleeds and hospital stays has a different outlook on life and different needs to meet than a teenager who goes through wanting to belong and significant peer pressure if being different, than a young adult who seeks independent life and success in his future projects.

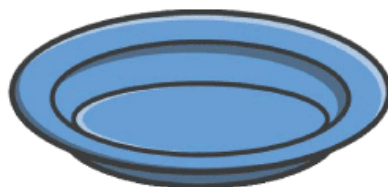
All of these different needs require different approaches, different resources and different stakeholders to be involved. It is essential for NMOs to be aware of those differences in order to be able to best support their members with inhibitors.

To find out the needs specific to the NMOs and the PWI present at the workshop, the participants engaged in a practical exercise, through which they found out the ingredients of a good patient programme of an NMO; the differences in needs of PWI compared to a person without an inhibitor and things that can be changed to accommodate the needs of PWI to be able to engage more fully with the work of the NMO.



THE INGREDIENTS OF A GOOD NMO PATIENT PROGRAMME

- Establishing a very good conversation with specialists
- Having a (good) link with HTC
- Understanding patients' needs
- Doing needs analysis, data, surveys about inhibitor community
- Building a very good internal connection between members, having social strength
- Providing good information, education, treatment etc.
- Providing opportunities to socialise and (learn) to advocate for oneself
- Integrating of different subgroups in NMO
- Having multidisciplinary programme
- Reaching out to patients that are not members yet
- Advocating for more treatment and new treatment options, including inhibitor treatment
- Having human resources, skills to organise, financial resources
- Being patient community led
- Having sufficient funding
- Knowing patients' needs and difficulties



THE DIFFERENCE IN NEEDS OF PWI COMPARED TO A PERSON WITHOUT AN INHIBITOR

- Access to treatment is much more limited
- Treatment is more tiring and exhausting
- The need for treatment is indispensable, but it is more expensive
- There is no prophylaxis for PWI
- The multidisciplinary programme for PWI is really essential
- There are disability and mobility issues
- The isolation is quite high
- The socio-economic issues are very present, e.g. underemployment
- There is a lack of focus from NMOs



THE THINGS THAT CAN BE CHANGED TO ACCOMMODATE THE NEEDS OF PWI

- As NMO:**
- Train one medical specialist (physiotherapist, dentist, pediatrician) to be specialised in inhibitors
 - Train PWI in advocacy to be able to liaise with medical specialists
 - Create a place to connect among PWI
 - Organise a meeting for PWI only
 - Create inhibitor-focused programmes
 - Ensure board involvement in inhibitor issues and vice versa
 - Have a PWI in the structures of NMO
 - Do advocacy about inhibitors (scientific + experiences of PWI)
 - Ensure equal treatment for PWI across Europe
- As PWI:**
- Have self-belief, self-confidence
 - Communicate inhibitor stories to the community
 - Become an expert patient or expert parent

SHARING GOOD PRACTICES:

SECURING TREATMENT WITH THE MOST EFFECTIVE AND EFFICACIOUS PRODUCTS INCLUDING NOVEL THERAPIES

During the workshop it was essential to address the idea that better inclusion of PWI in the work of NMOs is a **two-way process**. On one hand there needs to be a concerted effort from the NMOs, but on the other hand it is vital that PWI are pro-actively engaged in ensuring their own inclusion and promoting self-advocacy.

One of the cornerstones to self-advocacy is knowledgeability, particularly regarding the options available for oneself. The aim of this session was to explore one of the issues that is of high interest to PWI – ensuring treatment with the most effective and efficacious products, including novel therapies.

The session combined an input from a clinician - **Dr Paul Giangrande** - and experience brought from the NMOs and patients - **Mr Edouard Hamelin** (French NMO) and **Ms Teresa Pereira** (Portuguese NMO) who kindly shared their experiences.

Dr Giangrande stressed that inhibitors are still a significant issue and cause for concern for people with haemophilia. However, it is an exciting time in relation to the treatment of inhibitors in haemophilia patients. New novel and by-passing therapies, new methods of administration and the possibility for safer and more effective physiotherapy and surgery means that the quality of life for people with inhibitors can be greatly improved. Dr Giangrande gave an overview on the developments in relation to medical treatment of inhibitors but also the importance of the European Principles of Inhibitor Management (EPIM) that were published this year and serve as the basis a strong advocacy tool

for patients, clinicians and NMOs in order to access the safest and most efficacious treatment but also to assist in developing a framework for improved access to comprehensive care to deal with the complexities of living with a rare complication in a rare disease.

A major clinical landmark development this year was that one novel product was licensed for use in patients with inhibitors. There are other novel therapies and by-passing agents in the pipeline and the future is looking bright for treatment for people with inhibitors. While securing access to these agents may be a significant challenge

for patients, clinicians and NMOs throughout Europe the recently published EPIM will compliment this collective advocacy drive.

Dr Giangrande also pointed to the joint statement of EHC and EAHAD issued regarding the introduction of all novel therapies.

Dr Giangrande highlighted three **key pieces of information that are absolutely necessary to communicate to the patient before starting any novel therapy** 1) he noted that the long-term consequences are not explored yet as the product has been available for a short time;

2) that the patients should be careful not to overdo sports; and 3) that the interaction with other treatment products is not clear yet and together with their clinicians they should be aware of that.

Mr Edouard Hamelin shared his experience from the patient's perspective regarding a novel product, and Ms Teresa Pereira did so from the perspective of a mother of a child with inhibitors.





SHARING GOOD PRACTICES:

ACCESS TO SURGERY

The aim of this session was to explore one of the issues that is of high interest to PWI - access to surgery. The session featured input from a clinician – **Dr Paul Giangrande** - and experience brought from NMOs and patients through the presentation of **Mr Miguel Crato** (Portuguese NMO).

Dr Giangrande discussed the clinical aspects for people with inhibitors considering or requiring surgery. With improvements in treatment and continuing developments in the area of novel and by-passing therapies, the ability for patients to increase physiotherapy, undergo surgery and improve their quality of life is becoming a reality for patients with haemophilia with inhibitors. For people with inhibitors with significant joint damage, surgery can be a last resort when the patient is experiencing ongoing pain, which is the biggest factor for consideration in surgery. Another consideration for people with inhibitors considering surgery is that joint replacements have a time-frame of approximately 10 - 15 years depending on the joint and the patient's physical activity, therefore it is an important consideration that a patient's age, pain level and physical activity are monitored and discussed at length with their haematologist and orthopaedic surgeon before a decision is made.

Dr Giangrande also highlighted that despite the advancements in treatment, new by-passing agents for example will not be suitable alone for treatment during surgery. Conventional methods of treatment will need to be administered for patients during surgery. This alongside new therapies is a new era of treatment and will need to be discussed with the patient's haematologist prior to surgery. Dr Giangrande also highlighted

the realistic and very real keys to success for patients considering surgery, which were mental and physical preparation in the form of physiotherapy prior to and following surgery. It is also important that patients understand the effects of surgery; that a full range of motion will not be achieved but a reduction in pain and therefore improved quality of life are the ultimate goals. He also stressed the importance of the surgery being carried out under supervision of a multidisciplinary care team and in an expert centre.

Mr Crato shared his experience with his recent knee replacement surgery. He gave a detailed account of, how in close cooperation with his

haematologist and orthopaedic surgeon, the surgery was planned and executed.

Miguel stressed the importance of preparation for the surgery and the different aspects of this - multidisciplinary team meetings, achieving optimal dental care (to minimize the risk of infection), intensive physiotherapy to ensure proper strengthening of muscles, as well as mental preparation, ensuring the support of family, friends, and the workplace in performing day-to-day tasks after the surgery. He also noted that the patient should be prepared to have a very slow recovery after knee replacement surgery.

Further he discussed the practical aspects of the surgery, such as choosing the bypassing

agent, testing and the surgery itself, as well as post-surgery and recovery, especially the importance of physiotherapy.

A question was raised regarding the best age to perform knee replacement surgery, as the results seem to be quite good for patients and result in a significant improvement in the quality of life. Dr Giangrande pointed out that one would not wish to perform this surgery too early, especially not in a child, as a knee replacement does wear out eventually and one would have to do the surgery again. Therefore, timing is paramount – not too early, nor too late.



TOOLS FOR PWI INCLUSION IN NMOs: EHC MATERIALS AND HOW TO USE THEM IN PRACTICE?

In order to provide the NMOs with concrete tools for the inclusion of PWI and for PWI tools for self-advocacy, a session introducing available EHC materials was held.

EHC Community Programmes Officer Kristine Jansone presented three sets of materials that can be used for PWI advocacy and inclusion by the NMOs.

1 - NMO Pack

In order to help people with inhibitors across Europe to connect, share experiences, and build a community and support network, a set of materials to support the efforts of the NMOs to reach out to patients with inhibitors was created and sent out to NMOs. It included flyers and posters informing the reader about the different areas of work of the EIN. The EHC also offered to work on translation of these materials together with the NMOs, so that they can also be printed and distributed in the local languages.

The participants of the workshop gave their feedback and discussed how to improve and to best use these materials for optimum results. The suggestions included a proposal to split the multiple and sometimes dense materials among the different target groups within an NMO, e.g. adults with inhibitors, parents of children with inhibitors etc., as well as to involve volunteers in the translation of the materials where the NMOs do not have readily available resources and engage inhibitor ambassadors and parents.

Most importantly, it was noted that the number of NMOs attending the EHC Inhibitor Summit in Barretstown should be increased as this is where the real engagement of PWI happens. It was also mentioned that specialised brochures (e.g. on surgery) would be helpful.

2 - Poster on European Principles of Inhibitor Management

The objective of the poster is to explain and promote the 10 principles of inhibitor care and management that have been published in the *Orphanet Journal of Rare Diseases*. It is aimed at patients so that they become aware of key elements of inhibitor treatment and care, and have a readily available advocacy tool to use in discussion with relevant stakeholders regarding what they are entitled to. The poster will be sent to the NMOs and HTC for display in their public spaces where PWI can see and read it. The EHC has also offered to work on translation of these materials together with the NMOs, so that they can be printed and distributed in the local languages.

The outcomes of the discussion regarding the poster resulted mainly in suggestions and ideas on how to better distribute and use the poster, e.g. sharing the poster with the relevant Ministries of Health or creating a leaflet version for HTCs where poster display is not allowed.

3 - EIN Website

The website provides a space for people with inhibitors to seek information and resources, as well as discuss and share their daily challenges. A section dedicated to caregivers and parents offers information specifically tailored for them and a section dedicated to NMOs provides ideas, experiences and activity suggestions for them to implement within their own inhibitor communities. Visitors can find useful information about inhibitors, advice on day-to-day issues, travel and professional guidelines.

DEVELOPING TAILORED APPROACHES FOR PWI INCLUSION IN NMOs

The aim of the last session of the day was to exchange ideas and practices by working on real-life case studies from the experience of selected NMOs. The participants worked in groups to find the best ways to support PWI in the situations described in the case studies, such as how to establish a good relationship and trust with health care professionals and how to facilitate access for PWI to education and jobs.

The conclusions reached in the both of the cases reviewed by the participants were that **the most essential things to ensure that the needs of PWI are met from the variety of perspectives** are to:

1) provide relevant and consistent education for both patients, their caregivers and family members, as well as healthcare professionals;

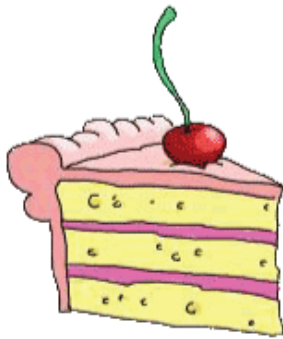
2) raise the standards of care wherever possible and maintain high standards;

3) ensure sufficient communication with all the stakeholders involved, including schools and employers. It is also important to gather tips and exchange experiences among the patients and NMOs.

CONCLUSIONS AND CLOSING

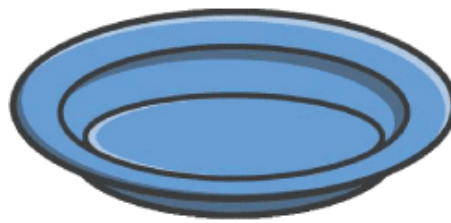
In the conclusion of the workshop the participants were asked, following the presentations and discussions during the day, if they would add something to the things that can be changed to accommodate the needs of PWI.

The additions are noted on the “post-it”.



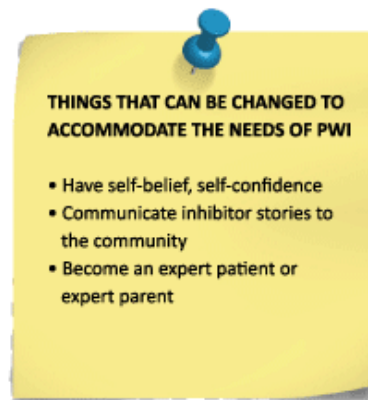
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A BIG THANK YOU TO ALL THE
CONTRIBUTORS, PARTICIPANTS
AND SUPPORTERS FOR A
FANTASTIC WORKSHOP!

