EUROPEAN RARE &
INHIBITOR
NETWORK

EXTREMELY RARE BLEEDING DISORDERS AND INHIBITORS

TREATMENT AND ACCESS FOR ALL BLEEDING DISORDERS

Brussels, Belgium

16 June 2023

ERIN

EHC
european haemophilia consortium
Advocating for people with haemophilia and congenital bleeding disorders

Mapping the needs of people living with extremely rare bleeding disorders

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Introduction to survey

Aim

To inform the building of the European Rare and Inhibitor Network.

Distribution

Sent to all the 48 NMOs of the EHC + HTCs and available in **2 languages –** ENG, RUS.

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Profile of respondents

- In total **34 valid responses** received.
- 8 of EHC National Member Organisations (NMOs) represented:
  - Belgium (2), Denmark (10), France (1), Germany (2), Latvia (1), Netherlands (8), Switzerland (6), UK (4).
- The respondents represented following **bleeding disorders**: Glanzmanns Thrombastenia, afibrinogenemia, Factor II, Factor VII, Factor XI, Factor XII, Factor XIII deficiency, platelet disorders.
- 2 **undiagnosed**!
- The respondents were **male** (13), **female** (19) and **other** (2).
- Age of respondents between 4 and 74 years.

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Participation & Community

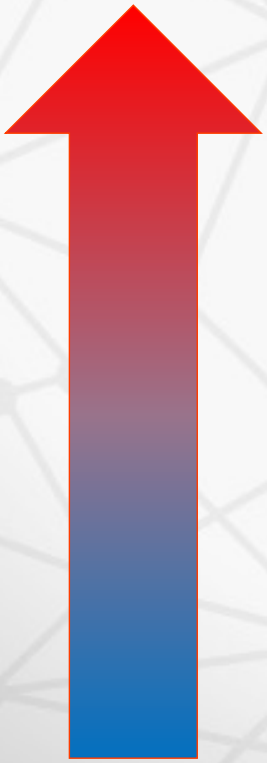
- **60 % do not participate** in the activities of the NMOs/other PO
- **Why?**
  - More structural meetings
  - All about haemophilia
  - No diagnosis
  - No impact of the bleeding disorder

"Different and sometimes alone. The meetings most of it are about haemophilia. The information is not really for me. But being with other bleeder is good to have. The more specific meetings are more informative. But still feel rare. Also within the rare you see so many different symptoms. It is not always easy to find the same solutions. But being bleeders makes the connection."

Currently faced challenges

1 - the most challenging; 5 - the least challenging

What is the biggest challenge that you face in your life as a person living with an extremely rare bleeding disorder?



- Lack of information regarding my bleeding disorder
- Isolation
- Psychological/emotional discomfort
- Limited access to treatment (including treatment options) and/ or difficult medical management
- Difficulty in participating in social and/or professional activities

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Currently faced challenges

1 - the most challenging; 5 - the least challenging

- Other challenges included **having access to proper information from haematologists** following diagnosing.
- No information in the general population, including ER and GPs!
- General information about what and if something is going on in research of feature treatment;
- Pain, bruises, mental impact;
- The mental load of the disease (thinking about the blood clotting disorder every day).

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Mapping the needs of people who have haemophilia with inhibitors: EHC survey for PWI

Introduction to survey

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- **Aim** - to better understand the situation of the people with haemophilia, the challenges they face and the possibilities for support.
- The **data yielded will be used to develop an optimal programme**, tailored to fit the needs of the target group mentioned above.
- Survey was carried out between 04/09/2015 and 11/01/2016 , sent to all the 45 NMOs of the EHC and available in **10 languages**.
- In total **208 responses** received, not always complete.

Profile

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- Survey was carried out between 04/09/2015 and 11/01/2016 , sent to all the 45 NMOs of the EHC and available in **10 languages**.
- In total **208 responses** received, not always complete.

- 28 of EHC NMOs represented:
  - Austria, Azerbaijan, Belgium, Bulgaria, Denmark, Finland, France, Germany, Greece, Ireland, Italy, Kazakhstan, Kyrgyzstan, Latvia, Lithuania, Macedonia, Moldova, the Netherlands, Poland, Portugal, Romania, Russia, Serbia, Spain, Switzerland, Turkey, United Kingdom, Ukraine
- Connection to **inhibitors** :
  - 104 are PWI
  - 79 family members/ caregivers
  - 21 other persons, such as haemophilia patients without inhibitors, volunteers in the NMO or healthcare professionals
  - 4 skipped this answer

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- **Gender:** 119 men and 80women. 9 skipped the question.
- **Age:**
  - **<18** – 10%
  - **18–30** – 20%
  - **30-50** – 43%
  - **>50** – 23%
  - skipped – 4%
- **Habitation** - out of 65% who responded, the distribution is following:
  - 49% - live with their own family
  - 25% - live with spouse
  - 7% - live alone
  - 17% - live with their parents
  - 2% - other
- **Geographical situation -** out of 60% who responded, the distribution is following:
  - 29% - small town
  - 22% - capital
  - 12% - village
  - 26% - other major city
  - 11% - countryside

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- **52% face mobility challenges**
  - Regular use of assisting devices: 26% of cases crutches, in 10% cases wheelchair and in 8% cases a cane
  - Occasional use of assisting devices:crutches (16%) and wheelchair (10%)
  - 15% - other mechanisms n place to assist their mobility, such as orthosis or lift installed in the house
  - 15% - no assisting devices
- **Languages**
  - 35% speak **only their own language**
  - 65% speak **at least one other widespread language** - EN(35%), IT(13%), DE(11%), RU(10%), FR(9%), ES(6%), PT(3%)
- **Internet access**
  - All the time – 85%
  - Once per day 11%
  - Sometimes – 3%
  - Rare – 1%

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Main challenges on daily basis

	1-5 points	6-10 points
Lack of Information about the condition	**59%**	**41%**
Access to treatment	52%	48%
Difficult medical management	**37%**	**63%**
Difficulty in participating in social activities	57%	43%
Difficulty accessing education/jobs	55%	45%
Stigma	**70%**	**30%**
Isolation	58%	42%
Psychological/emotional discomfort	50%	50%
Challenges in every day activities (e.g. household)	**31%**	**69%**
Difficult financial planning	51%	49%

- **Other:**
  - **medical** (dental surgery and other medical invasions, lack of treatment and prophylaxis, absence of cure)
  - **systematic** (treatment costs linked to financial planning, lack and ignorance of healthcare professionals specialized in haemophilia and inhibitors)
  - **personal** (no hope or no control over the condition, mobility challenges and pain, lack of understanding in the society) challenges mentioned

ERIN Manifesto

The process

Co-creation process during the ERIN Summit in December 2022.

The result

Signed document outlining needs that they have as a community affected by haemophilia with inhibitors and extremely rare bleeding disorders, the challenges they face in having those needs met and support that they require to have this needs met on European and national levels.

Aim

To inform the work of the ERIN committee

ERIN Manifesto

We are people affected by haemophilia A with inhibitors, haemophilia B with inhibitors and extremely rare bleeding disorders, such as clotting factor V, VII and X deficiency, who gathered at European Rare and Inhibitor Network (ERIN) Summit in Ireland from 1-4 December 2022. During the Summit we had an opportunity to come together in numbers that we cannot meet in our local context due to the rarity of our condition. We learned from multidisciplinary care experts, and most importantly – from one another. Together, we outlined needs that we have as a community affected by haemophilia with inhibitors and extremely rare bleeding disorders, the challenges we face in having those needs met and support that we require to have those needs met on European and national levels.

Extremely Rare Bleeding Disorders
We are overlooked as patients and neglected by healthcare professionals, healthcare systems and government. Due to our bleeding disorders, we face challenges in our personal and professional lives that urgently need to be addressed.

Our most urgent unmet needs are access to better treatment, accurate diagnosis, and well-informed, dedicated doctors that focus specifically on extremely rare bleeding disorders. We need to enhance the solidarity for people affected by extremely rare bleeding disorders within and outside the bleeding disorders community.

To be able to achieve this we need to be empowered and independent, feel included, gain knowledge and have access to appropriate information. We need the support from our direct social network – family, friends and colleagues as well as our National Member Organisations and the EHC, as well politicians and industry and other relevant stakeholders.

Haemophilia B with Inhibitors
We are people and families with haemophilia B and inhibitors who face the expected and more importantly – the unexpected every single day. We live with the pain and damage that has been done to our bodies due to the lack of adequate treatment, we live with missed education and job opportunities.

Our most urgent unmet need is access to new prophylactic treatment, as well as adequate multidisciplinary care, especially tailored physiotherapy. We need systematically more time with our doctors, as well as more doctors that focus specifically on rare bleeding disorders. We need more awareness and education about bleeding disorders outside our community – in kindergartens, schools, universities.

To be able to achieve this we need support from our direct social network – family and friends, we need our children to be our guides. We need nurses and doctors to treat with the best options available and to be educated on the latest scientific developments in bleeding disorders treatment, as well as to work in close cooperation among all healthcare specialists – haematologists, physiotherapists, dentists, surgeons, psychosocial experts, nurses etc.

Haemophilia A with Inhibitors
We are a strong community of people affected by haemophilia A with inhibitors that have needed considerable resilience in our path. We have been very isolated and underserved for a long time and lived with limitations in our wishes and desires. Even though significant progress has been made in terms of the treatment that we have, there are many challenges that remain and work needs to be done to maintain the progress and the improved quality of life that has been recently achieved.

We need to ensure that everyone affected by haemophilia A and inhibitors is living a pain-free life and that there is accessible personalised care for everyone, as well as holistic view on haemophilia treatment and access to multidisciplinary care, regardless of where you live. We need a tool to better assess the effects of treatments and interventions.

To be able to achieve this we need a mindset of solidarity and thinking beyond oneself, as well as the ability to transmit the knowledge gained to our local communities. We also need health care providers that are dedicated to acquiring all available knowledge and sharing it with the patients as equal partners. We need standardised policies on bleeding disorders around Europe, as well as high level of advocacy and collaboration between the stakeholders.

We ask that the EHC, our National Member Organisations and the ERIN Committee who will start work on the European Rare and Inhibitor Network in 2023 take this document into account when planning their work.

We ask that in order to improve the treatment and care for all of us, multi-centre research is carried out and new approaches to data collection and treatment development are found.

We ourselves commit to being actively involved in the process of meeting our needs and the needs of those affected by the rare bleeding disorders in our countries, especially those who find themselves outside the bleeding disorders community and the future generations to follow, so that no one is left behind.

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What would you add?

What is challenging for you?

What is your most urgent unmet need?

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Let's discuss!

What are the roadblocks that we are facing

to have he treatment and care that is needed?

On the level of healthcare system?

On the level of the NMO?