

Introducing: European Inhibitor Network (EIN)

Amanda Bok
CEO

European Haemophilia Consortium

About EHC

- European patient organization (umbrella)
- Founded 27 years ago
- Haemophilia, von Willebrand Disease, other Rare Bleeding Disorders
- 45 National Member Organisations (NMOs)
 - 27 EU Member States + Council of Europe countries
 - EHC NMOs = WFH European NMOs
- HQ in Brussels

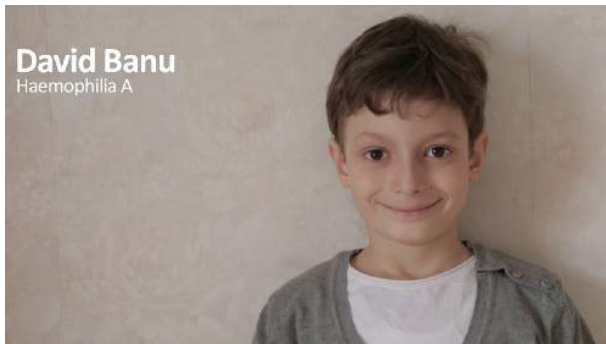
Main strategic objectives 2014-2017

1. Support, empower NMOs
2. Promote access to optimal treatment and comprehensive care for people with RBDs
3. Engage constructively w/all key stakeholders
4. Influence relevant European policy-making

EIN - background



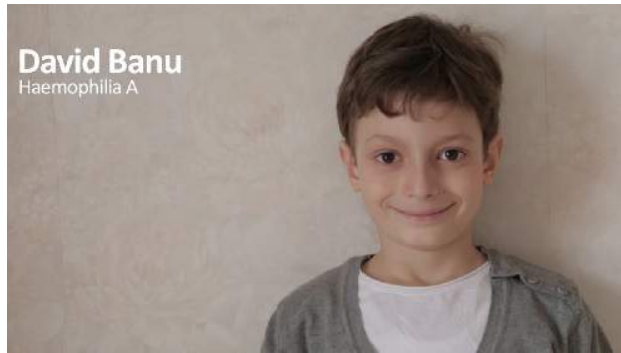
- Considerable and **underserved** community
- Very **isolated** group of patients
- **Small numbers** nationally
- Significant **challenges** – personal/systemic
 - Lack of information, insufficient treatment, mobility constraints, social and financial barriers, psychosocial impact, etc.



- 8 years old
- 2 years old when diagnosed w/ inhibitors
- No inhibitor treatment
- 6 years old when started ITI – charitable donation
- Good QoL but strained budget – travel abroad



- 15 years old
- 10 years old when started haemophilia treatment
- No inhibitor treatment
- Limited emergency care/ pain medication, compress
- Reduced mobility and challenging family life



- *“When he was three years old he asked me if I could cut his arm off and give him a new one so it wouldn't hurt.”*
- *“I'm afraid of the moment when the donation is going to run out.”*
- *“There is no treatment even when there is an emergency, ... we fixed the bleed with painkillers and compresses and it lasted 3 weeks.”*
- *“We got a paper, which said that he had reached his cost-limit since he has had too many treatments.”*

EIN - objectives



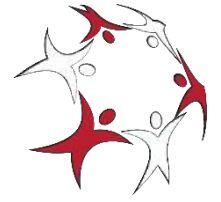
1. Improve **understanding of needs**
 - And how to meet them
2. Build a **community** of PWI and their caregivers
 - Allowing mutual support, education, empowerment
3. Provide education, advocacy training
 - To **engage with decision-makers**
4. Work with medical experts
 - **Towards framework** for treatment and care

EIN - elements



- Needs analyses/surveys
- Face-to-face meetings
 - Summit and workshops
- Online community
 - Support, education, exchange
- Educational materials
- Advocacy tools
- Treatment and care – towards a framework
 - Initially 3-year program (2015-2017), renewable
 - Translation integrated as much as possible

3 needs assessments



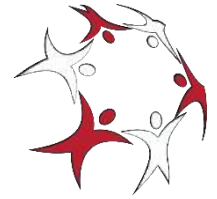
- Clinician survey
 - To be sent soon
- NMO survey
 - Completed (but open for ongoing feedback)
- Patient survey
 - Completed (but open for ongoing feedback)

1: NMO survey - objectives



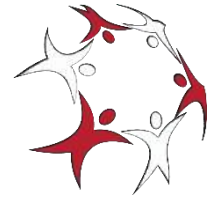
- To better understand position /condition of PWI within their NMOs
- To map NMO initiatives and projects for PWI, family and caregivers
- To tailor the EIN to NMO gaps/needs
- **26 / 45** ($\approx 60\%$) EHC NMOs responded
- Survey closed but NMOs invited to continue feedback/contributions

Numbers

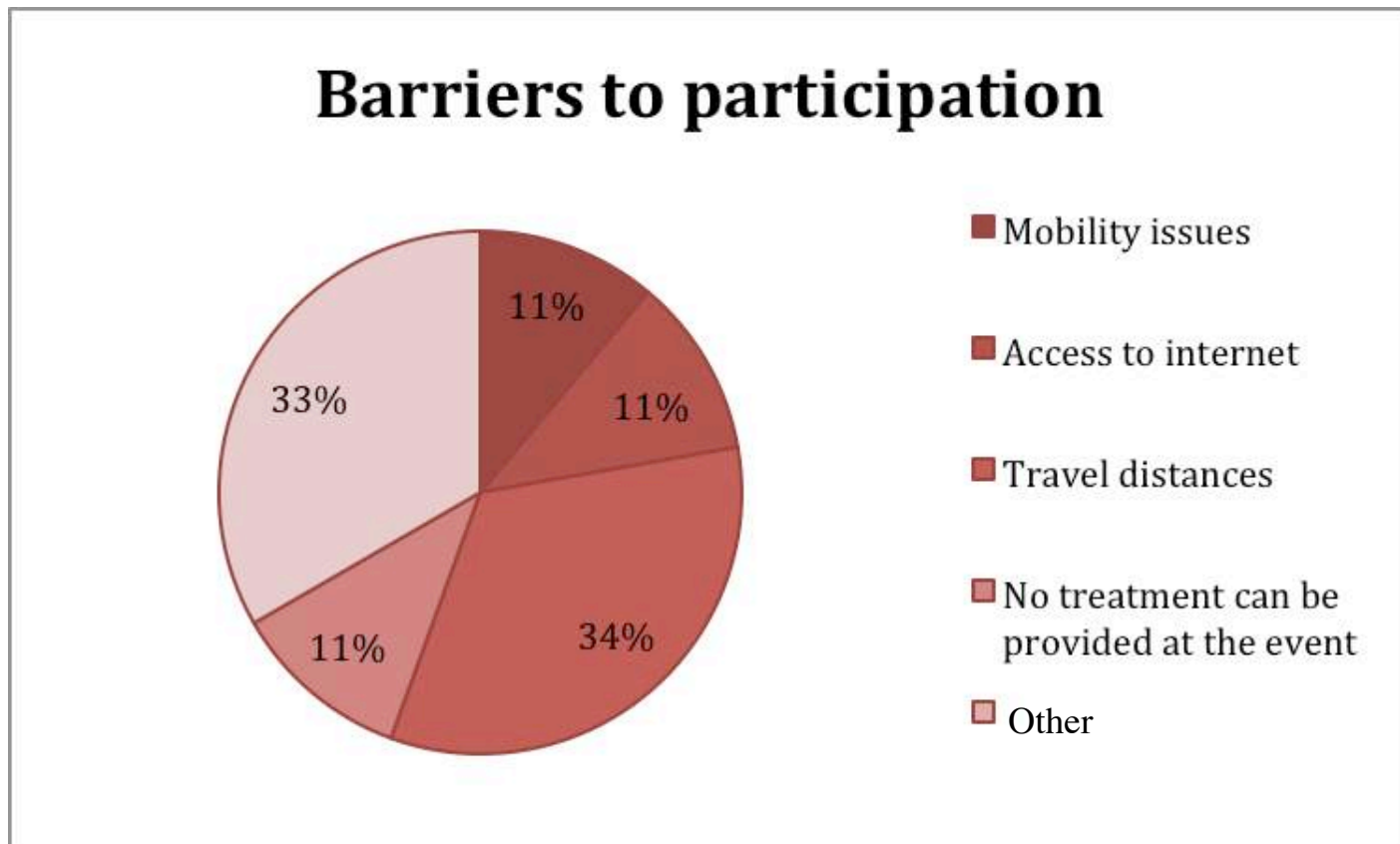


- **163 people with haemophilia A** have inhibitors
- **10 people with haemophilia B** have inhibitors
- Not all NMOs had access to this information (admin reasons)
- Haemophilia A: between **1-64 people/country** have inhibitors
- Haemophilia B: between **0-3 people/country** have inhibitors
 - NMOs face very different situations in their communities, this impacts what they offer PWI, resources they can allocate and special materials they can develop/provide
- **Age:** <2 (7%); 2-11 (23%); 12-18 (18%); adults (52%)

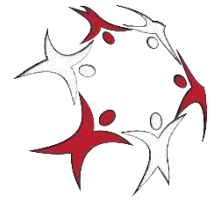
Participation



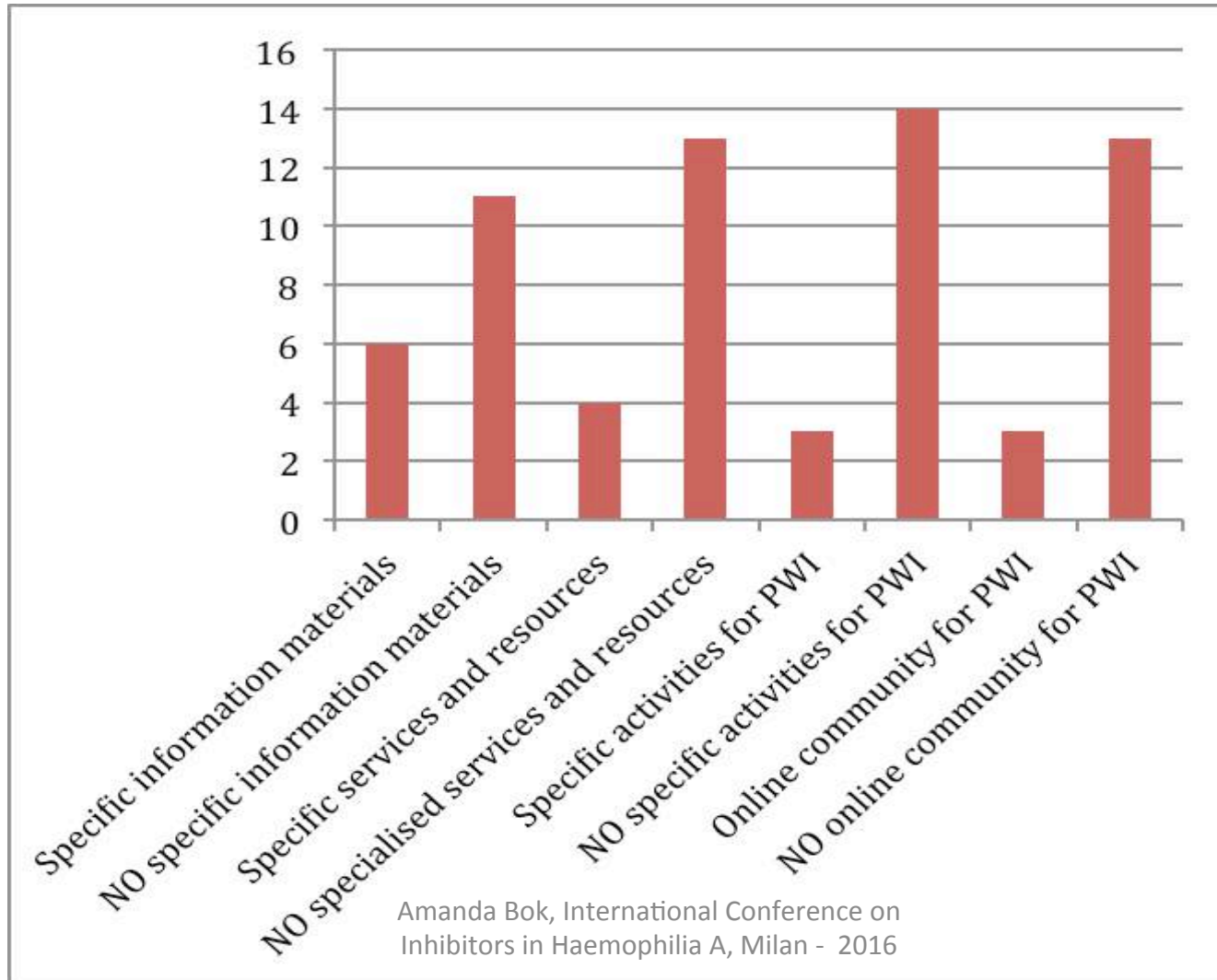
- PWI generally participate in NMO activities
- Only 2/17 NMOs who responded to this question indicated that PWI do not participate in the activities of the NMO

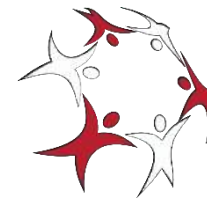


Specialized materials & activities



- Lack of specialized products = small numbers





What exists in NMOs

- Only 4 NMOs from respondents offer **specialized resources**:
 - medical advice
 - social/psychological support
 - web resources
 - telephone helpline upon request in NL
- Only 3 NMOs from respondents offer **specialized activities**:
 - orthopedic issues
 - special camps
 - educational seminars
 - special physiotherapy courses
 - In 1 NMO other activities available through CCC/HTC
- Only 4 NMOs have **specialized events for family and caregivers**
 - special educational courses (e.g. physiotherapy)
 - psychological support
 - workshops for parents
- Only 3 NMOs have **online communities** for PWI

Information materials



- **Need materials on :**
 - Latest information about inhibitors
 - Treatment information
 - Bypassing agents, ITI, protocols
 - Diagnostics
 - Surgery
 - Clinical trials of new medicaments
 - Ageing
 - Case reports from people with inhibitors

Specialized resources



- **Potentially needed resources and services:**
 - Medical advice
 - Psychosocial support, both for patients and parent
 - Mobile phone applications
 - Availability of database to improve quality of life
 - Blogs
 - Annual European newsletter on research and cases
 - Peer support
 - Ergotherapeutic advice
 - Links from the websites of other NMOs and web resources
 - Web-based information that could be translated into the languages of the NMOs

Main NMO challenges



NMOs indicated the following main challenges in their efforts to support PWI:

- Small number of people
 - E.g. limited resources, large travel distances
- Lack of communication and information
 - E.g. international, limited exchange of information among different countries
- Conclusion: welcome European level initiative

2: Patient survey



- Open for approx. 5 months and available in **10 languages**
- **208 responses received**, not always complete:
 - 104 PWI
 - 79 family members/caregivers
 - 21 other persons, such as haemophilia patients without inhibitors, volunteers in the NMO or healthcare professionals
- **Age:**
 - **<18** – 10%
 - **18–30** – 20%
 - **30–50** – 43%
 - **>50** – 23%
- The survey will remain open for ongoing feedback

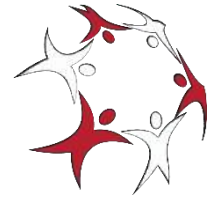


Main challenges on daily basis

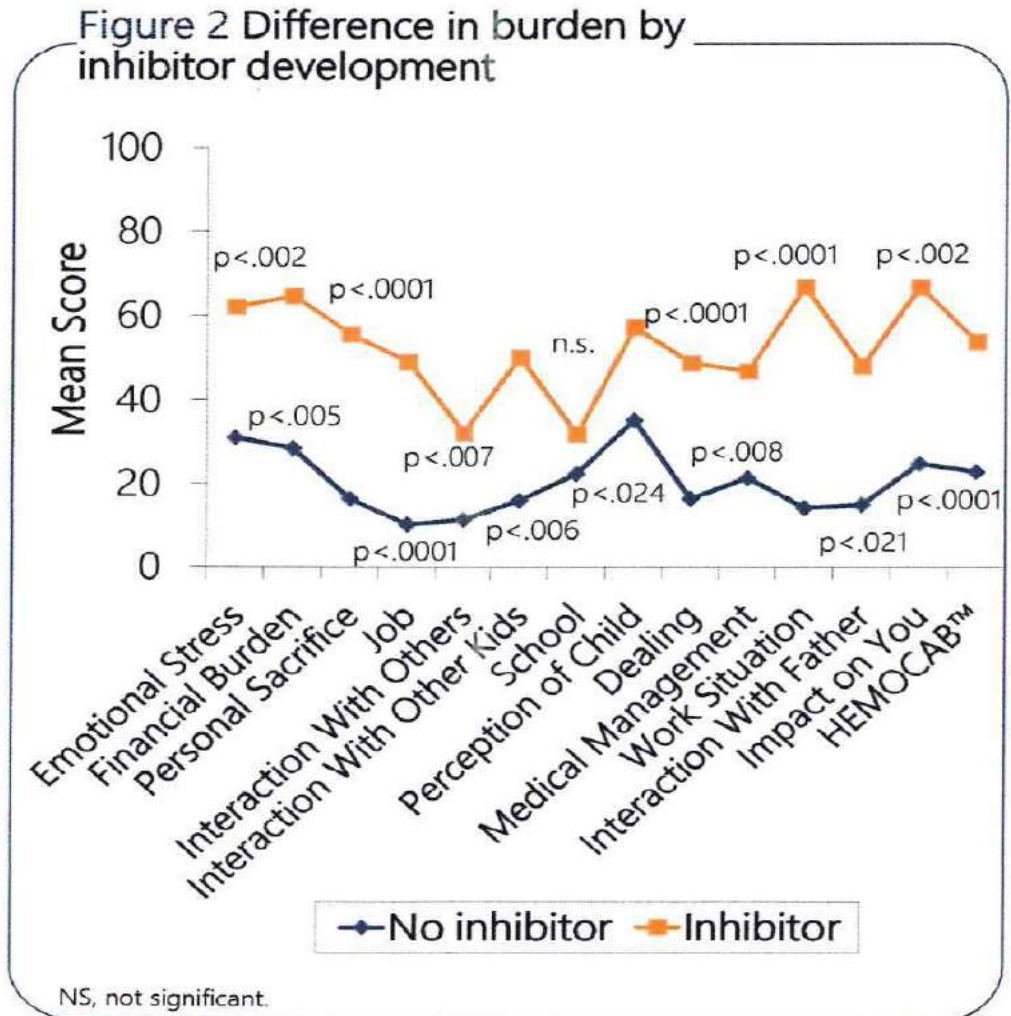


	1-5 points	6-10 points
Lack of Information about the condition	59%	41%
Access to treatment	52%	48%
Difficult medical management	37%	63%
Difficulty in participating in social activities	57%	43%
Difficulty accessing education/jobs	55%	45%
Stigma	70%	30%
Isolation	58%	42%
Psychological/emotional discomfort	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		

Psychosocial impacts



- Main challenges for caregivers/ family:
 - Perception of a child
 - Emotional stress
 - Financial burden
- **Increased substantially** by the presence of an inhibitor
- Main challenge for immediate social environment – **lack of information**



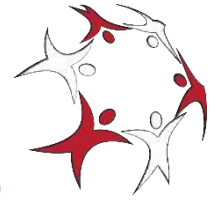
Areas needing support



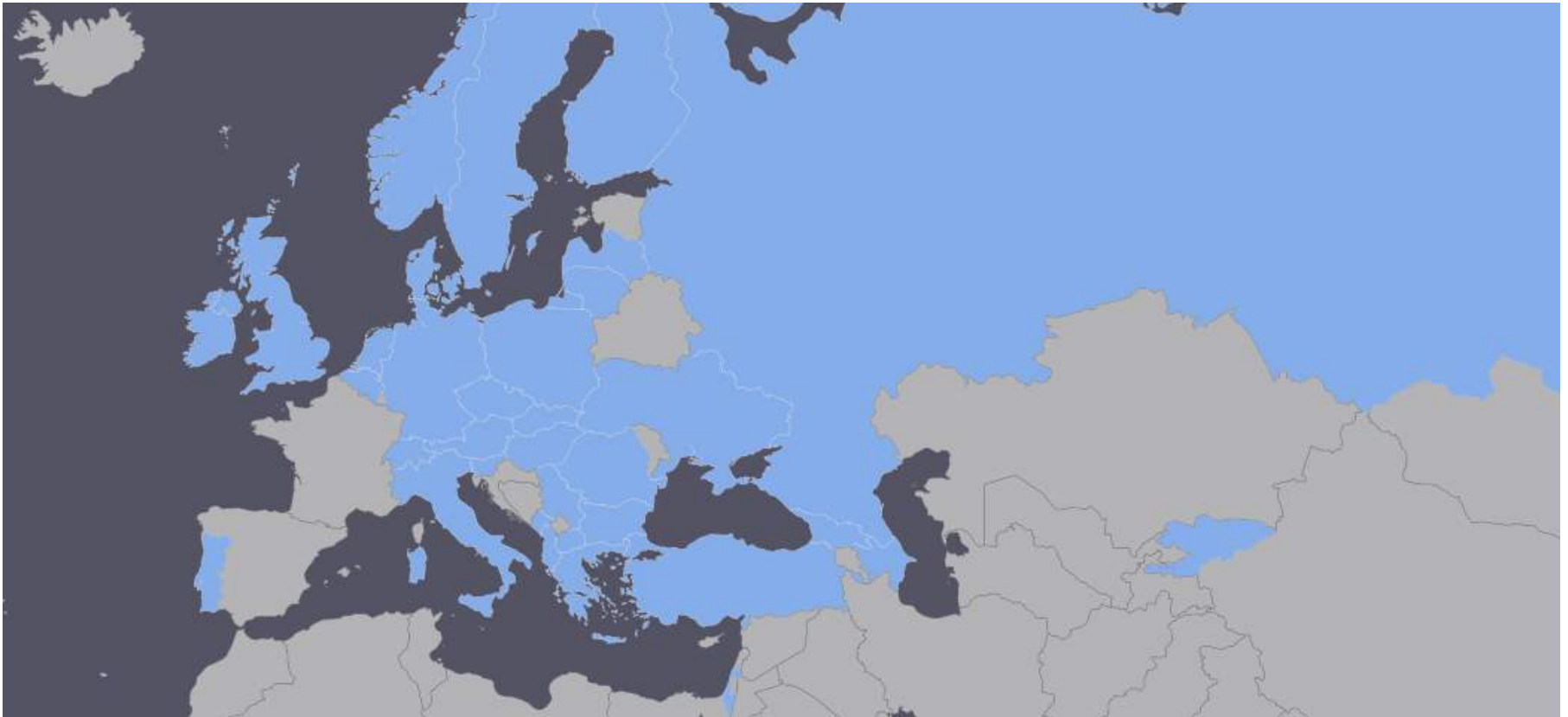
- The **most needed support** is:
 - Medical support (23%)
 - Information, publications and media (13%)
 - Networking and peer support (13%)
 - Financial support (11%)
- The **most important themes to address** would be:
 - Latest information on treatment
 - Expert advice on strategies for improved quality of life
 - Experiences and stories from peers
- Most respondents want to actively participate in an online community
- All respondents want activities at European level

2015 EHC Survey

Haemophilia Care in Europe



- Preliminary results
 - Respondents to-date in blue



34 respondent countries



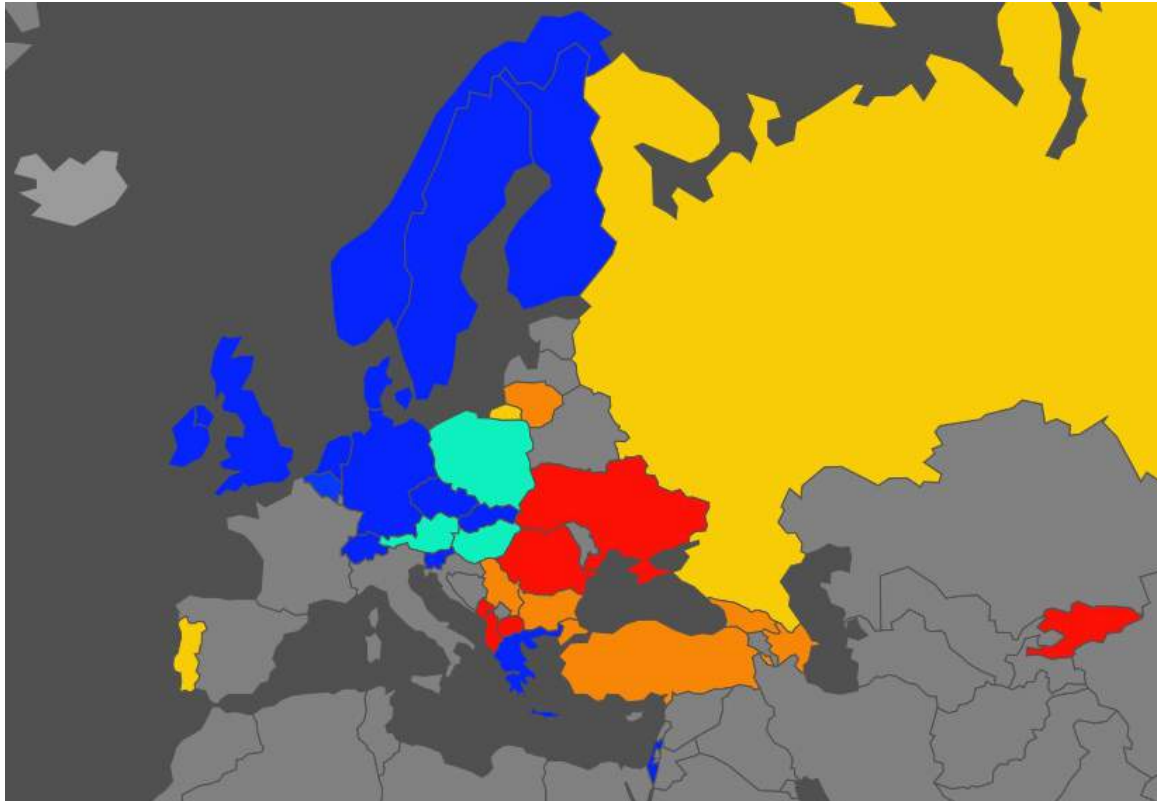
- Albania
- Austria
- Azerbaijan
- Belgium
- Bulgaria
- Czech Republic
- Denmark
- Finland
- Georgia
- Germany
- Greece
- Hungary
- Ireland
- Israel
- Italy
- Kyrgyzstan
- Latvia
- Lithuania
- Macedonia
- Montenegro
- Netherlands
- Norway
- Poland
- Portugal
- Romania
- Russia
- Serbia
- Slovakia
- Slovenia
- Sweden
- Switzerland
- Turkey
- Ukraine
- UK

What did EHC ask about inhibitors?



“What percentage of patients developing inhibitors have access (where appropriate) to immune tolerance induction therapy (ITI)?”

Overview of responses



Percentages of patients with access to ITI

100% – **76 to 99%** – **50 to 76%** – **1 to 25%** – **0%**

Treatment and care



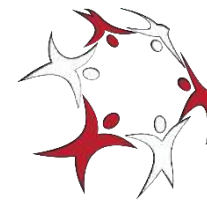
- Need to ensure optimum treatment and care, regardless of the geography of PWI
- Need to work towards agreed frameworks/principles
 - Several guidelines for treatment and care exist (e.g. Sweden, UK) but need to standardize/bridge differences
 - Specific standards also needed on:
 - Testing
 - Registries
 - Access to and choice of treatment products
 - Tailored comprehensive care (incl, families/caregivers/job counseling)
- Need to engage with all stakeholders

Governance



- European Inhibitor Program Officer
 - Kristine Jansone
- Inhibitor working group
 - PWI, parents, caregivers, HCPs, advisor
 - Prof Paul Giangrande, Chair (UK)
 - Miguel Crato (Portugal)
 - Carl Fredrik Gustavsson (Sweden)
 - Mirko Jokic (Serbia)
 - Elisabeth Olesen (Denmark)
 - Hannele Kareranta (Finland)
 - Dr Oleksandra Stasyshin (Ukraine)
 - Radoslaw Kaczmarek, EHC Steering Committee
 - Brian O'Mahony EHC President
 - Prof Flora Peyvandi, EHC MAG
 - Amanda Bok, EHC Office
 - Christina Burgess, Ex-Officio member (UK)





Thank you!