

World Haemophilia Day - Interview with Amanda Bok (EHC)

The 30th anniversary of World Haemophilia Day helps to illustrate the importance to support the bleeding disorders community. Read our interview with Amanda Bok, CEO of the European Haemophilia Consortium (EHC) & Co-Chair of EuropaBio's Patients BioForum.

This 17th of April marks the 30th anniversary of World Haemophilia Day. Could you please tell us more about haemophilia and the activities of the European Haemophilia Consortium that you are heading?

Haemophilia is a rare bleeding disorder in which one of a series of clotting factors is either missing or functioning in a deficient manner, thereby causing prolonged and dangerous bleeding, which has damaging short- and long-term effects, leading to severe disabilities, and sometimes death. However, despite having this one type of bleeding disorder in our name, the European Haemophilia Consortium (EHC) actually represents much more than just haemophilia; in fact we represent more than a dozen congenital rare bleeding disorders, most of which are less well-known than haemophilia but of equal or greater consequence to the safety and/or quality of life of patients.

How many patients are there in Europe?

We know there are more than 100,000 people affected by bleeding disorders in Europe. However, that number is a gross under-estimation because in many bleeding disorders we still face challenges with adequate diagnosis and reporting, and therefore many affected individuals remain invisible. The prevalence of different congenital bleeding disorders varies in range from 1 in 10,000 people to 1 in 3 million people, approximately, depending on the disorder.

What therapies are available?

Haemophilia has the most therapies available of any rare bleeding disorder or quite possibly of any other rare disease, which is a privileged position to be in compared to our orphan peers, however still remains nowhere near the quality of life, treatment and care of a 'mainstream' chronic disease. The good news is that right now we're currently witnessing a boom of innovation in the haemophilia treatment landscape and a plethora of very different and very exciting therapies may soon be coming to the European market. The bad news

is that such innovation is not paralleled in our other bleeding disorder areas, nor will access to these promising new haemophilia treatment products come easy for every haemophilia patient in every European country. Adaptability will be key, for all stakeholders involved in the healthcare system, particularly in our coming post COVID-19 future.

Which are in your view the most promising treatments for haemophilia patients in the pipeline?

All of them are promising, as is the fact that you're asking this question in a rare disease area. It is promising that we even have a *plurality* of treatment choices to consider. This is how it should be to ensure that we get to a future where the right treatment is given to the right patient for the right reasons at the right time(s) of their life. To help that happen, the EHC issues a regular review of all novel treatments and will soon launch an educational App to help patients (and other stakeholders) navigate their new and rapidly evolving treatment landscape. Hopefully, such promising variety of treatment choices will follow for all bleeding disorders and indeed all rare diseases, in the not-too-distant future. It should be our collective imperative to rephrase that question to '*What are the most promising treatments for rare bleeding disorders in the pipeline?*' and work backwards to its point of relevance.

What are currently the unmet needs of haemophilia patients and what has to still be achieved to enable easier access of patients to available therapies?

Haemophilia patients are a heterogeneous group both across and within European countries and face different levels and types of unmet needs. Not one European country complies with all 10 European Principles of Haemophilia Care, despite having more than a decade to implement them, and across our European region we continue to face huge disparities in access to adequate treatment. Gaps in adequate support prevail as well between disorder severities (with more resources being put towards severe patients than mild or moderate ones); genders (with haemophilia persistently seen as a male-only disorder to the unforgivable detriment of women with haemophilia); age groups (with a healthcare system unequipped to manage ageing patients with a rare disease); and response type (with patients who struggle with persistent treatment antibodies being further isolated and under-served particularly in less developed healthcare systems); to name just a few unmet needs. Easier patient access to available therapies requires a series of political and systemic changes. A strong step in the right direction would start with patient co-decision-making at national levels, including in the procurement and management of treatment products.

How is COVID-19 affecting haemophilia patients?

Ours is a community whose medically-prescribed treatment products infected many not once, but twice, with viruses - namely HIV and hepatitis C. So of

course already early on, when the Coronavirus was still limited to Wuhan, this community was closely following its evolution and staying connected both with each other as well as with trusted sources of scientific and patient information. One of their earliest questions naturally regarded the transmissibility of this new virus through the blood supply and in particular through therapies derived from donated blood products such as plasma (the answers-to-date are reassuring). As the scope of impact expanded, however, so did the questions. They turned inward to assess the vulnerability of this particular community to this particular virus, and to address changes in treatment plans and behaviours. Then, as global infrastructures became impacted, the questions turned outward toward international supply chains, manufacturing and delivery. As EHC we've worked hard to keep the community connected to us and to each other, which we've achieved so far through regular community-wide Zoom calls. Some aspects we're witnessing are positive: 1) Haemophilia and indeed other bleeding disorder patients are not *more* at risk of contracting COVID-19; 2) this community has a robust dose of resilience and strong coping mechanisms already built-in; and 3) the new clinical behaviours necessitated by COVID-19, such as patient-clinician co-decision-making and telemedicine, are to some extent already familiar practices in this community. Yet, other aspects will require special attention and care: 1) Despite not being *more* likely to contract COVID-19, *if* infected, the management of this virus can be more complicated in our patient community, particularly if immune-compromised (due for example to the aforementioned or other co-morbidities) or if patients are on certain types of therapy; 2) the baseline safety of patients may be negatively impacted by treatment obstructions such as through insufficient supplies, then further aggravated if as a result they need to visit a hospital; and 3) in the post COVID-19 environment, ensuring patients' access to the promising era of new treatments we discussed above - which was already going to be arduous for healthcare systems to absorb - will require *much more creative and innovative thinking by all stakeholders* than before COVID-19.

What should future research and innovation in bleeding disorders be oriented to in your opinion?

Just that: other bleeding disorders. It's a short answer with a long, overdue shadow.

The theme of World Haemophilia Day in 2020 is "Get+involved". How is the EHC marking this day?

First let me explain that the EHC doesn't mark World Haemophilia Day, April 17th, on the day itself out of deference to our national member organisations and their own, national commemorations. Instead, we typically mark it before or after. This year we planned to host a blowout event on April 24th as the closing of our 30th anniversary year (which we opened exactly one year ago)

and inauguration of our new office (which is our first and hopefully permanent home) by celebrating the fantastic engagement of our community.

For this we planned to unveil an interactive installation we've designed that lets viewers quite literally 'Open the Door to Another Reality...', namely to the many different realities of our different community members across time, age, gender, geography, family position, and disease type. Built to be immersive and engaging, we wanted to capture these patient stories during exactly this time of treatment transition, to remind not just patients but also to educate all stakeholders about what was involved to get 'here,' and will still be involved to get the full community access to adequate treatment and care.

Not to fear, we also wanted the installation to be mobile, to travel from member to member organisation, and to continue 'living' by gathering new and different stories over time. Therefore it will be easy to transport it to our EHC Conference in October for unveiling there, and then send it onward to its first national host.

So back to now, instead of one big World Haemophilia Day event on April 24th, we'll be delivering World Haemophilia Week instead, releasing one virtual programme element for each day of the April 20-24 week, covering different segments of our original programme, and delivered through multiple media to our community and the public. It's not as immediately exciting, there won't be any waiters and certainly no hugs, but I'll still have my glass of champagne at-the-ready and toast virtually to the health and prosperity of each and every member of our vibrant community.



Amanda Bok

Amanda Bok has been CEO of the European Haemophilia Consortium (EHC) since late 2012. Amanda first became involved with haemophilia when she joined the World Federation of Hemophilia (WFH) in 2009 in a communications capacity and later managed their international advocacy program. Before this, Amanda focused her efforts on international humanitarian aid and human rights issues, particularly child rights, in times of armed conflict. She worked for the International Committee of the Red Cross (ICRC) in Geneva and New York.