EHC NOW!

Seven things we learned from the EHC Round Table on 'Switching from standard therapies: where do novel therapies fit in?'

By Laura Savini, EHC Public Policy Officer

On Tuesday 22 November 2018, the European Haemophilia Consortium (EHC) held its third and last Round Table of Stakeholders of the year at the European Parliament in Brussels. The event was kindly hosted by Dr Cristian-Silviu Bușoi (EPP/Romania) and attended by Dr Miroslav Mikolášik (EPP/Slovakia), both long-standing supporters of the European bleeding disorders community. Dr Dan Hart, member of the EHC Medical and Scientific Advisory Group (MASAG) and Honorary Consultant Haematologist at Barts and the London School of Medicine and Dentistry chaired and moderated the event.

The topic of the event was 'Switching from standard therapies: where do novel therapies fit in?' Speakers tried to tackle the issue of exactly how clinicians and patients should decide when to switch from as standard half-life (SHL) coagulation factors to a series of novel therapies.

1. It all starts with education

Across all presentations speakers noted that clinician and patient education is key to ensuring that the correct decision is taken in relation to treatment regimens. Education should not be limited to the treatment options available but should be extended to having a clear understanding of how these medicines work and how they impact patients' health. Patients should be encouraged to learn about concepts of pharmacokinetics such as peaks and troughs as well as 'time under the curve.' In fact, clinicians believe that when patients understand how the treatment works and impacts them, they become more motivated to adhere to their treatment regimens and this will ensure better outcomes on their quality of life.

In addition, patients should be their very own advocates and be able to explain their treatment regimens and conditions to non-specialist clinicians in, for example, cases of emergency or when consulting non-haematologist health care professionals.

2. Personalisation and intensification are (or should be) the new normal

Novel therapies offer unique opportunities for the personalisation of treatment. This should not mean the 'rationing' of treatment but rather its intensification to achieve higher protection based on the individual patient's metabolism, lifestyle and other characteristics. This may mean maintaining the same infusion schedule on extended half-life coagulation factors (EHLs) but using it to achieve higher trough levels thereby having better protection against bleeds. This is particularly interesting for patients with more severe bleeding phenotypes, those that wish to engage in more active lifestyles or those with bad joint damage.

3. Non-linear progression of treatment innovation

The progression of haemophilia treatments from blood components to replacement therapies and beyond has moved exponentially. Viewing this progression as linear, however, implies superiority between the existing and novel therapies, which may be a misleading analysis. Instead, speakers

proposed that we now have a menu of treatment possibilities, options and choices for the real personalisation and individuation of treatment.

Of course, the reality is that in many countries novel therapies are not yet available, and in some they are available but only for a limited number of patients. Speakers stressed that SHL therapies remain a very safe and effective treatment and should still be considered even when novel therapies are available. Participants were also reminded that when launched, most EHL therapies had a similar price as SHL products, enabling some clinicians in some contexts to switch in order to achieve higher protection while using the same treatment budget. However, should SHL considerably reduce in cost, this would allow other reflections and choices to open up for clinicians and patients, for example enabling countries with limited health care budgets to provide increased access to treatment for their haemophilia patients.

The arrival of bispecific antibodies has been particularly innovative for haemophilia A patients with inhibitors who were previously not able to benefit from prophylaxis due to the short half-life of bypassing agents. However, it is important to note that bypassing agents will still be needed especially to treat acute bleeds and during surgery.

It is clear that we are entering a paradigm shift in the way we, as patients and clinicians, approach and consider treatment strategies and choices over their lifetimes. Increased personalisation will be the way of the future and will ensure that patients get the best outcomes from different treatment products.

4. Moderate haemophilia patients need more attention

Speakers noted that at the moment people affected by moderate haemophilia (i.e. patients with coagulation factor levels between 1 and 5 per cent) are the patients with the worst health outcomes. This is because in most countries these patients are not as prioritised as their severe counterparts and consequently are not provided with prophylaxis or home treatment and often face negative impacts on their joints. It is ironic therefore that, with the intensification of treatment, patients with severe haemophilia will effectively become either moderate or mild patients in the future. Therefore, isn't it time that patients with moderate (and even mild) haemophilia are also offered the same treatment opportunities? This is certainly a topic for discussion that, we foresee, will be recurrent in the coming years.

5. Patients need to be involved in the decision-making process
With all this innovation, the importance is increasing for patients to be actively consulted and involved not only in relation to their treatment regimens but also in the broader decision-making process on the organisation of haemophilia care and, in particular, the purchase of haemophilia treatments. With such different modes of action, novel therapies will be increasingly difficult to compare to each other and to standard therapies. This will result in challenges with assessing benefits and values. Most certainly new methods of analysis will be needed, said speakers.

6. Monitoring is needed
At the end of the day, these are novel therapies for which we still do not have much real world evidence with regard to safety and efficacy. Only real world clinical practice will give us more information on when these products are best used and their real safety profile. Therefore speakers recalled the importance of regularly monitoring patients on novel treatment, not only for inhibitors, and sharing this information in the treatment community.

7. Continuing European advocacy
In the concluding remarks, Dr Hart noted that as these treatments are not yet available everywhere and, as some countries still do not meet the minimum standard of haemophilia care, it is important to continue strong advocacy work in Europe.
In this context, the EHC's PARTNERS programme (Procurement of Affordable Replacement Therapies - Network of European Relevant Stakeholders) remains of high importance as it seeks to increase access to treatment in countries where European consensus standards are not yet met. The PARTNERS programme was launched one year ago in the European Parliament and will release its one year progress report and reflective White Paper shortly.

The EHC team hopes that this short 'hot-off-the-press' overview of this week's Round Table was useful. Stay tuned for a more detailed final report coming soon. The next Round Table of Stakeholders will be held in February 2019 on the 'Future of Comprehensive Care and Outcomes.' Follow us on our social media channels for more information.