

## EHC Round Table on Economics and Access, Health Care Systems and Novel Therapies

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Novel haemophilia therapies are undoubtedly transforming the current treatment landscape into an opportunity for better health outcomes for patients but also into an area full of unknowns. If there are cost differences between standard half-life (SHL) and extended half-life (EHL) FVIII treatment and it is possible to achieve the same trough levels with both – which will be more economically valued? If increasing the dose of SHL products can mean higher trough levels but EHL products bring the promise of less infusions – are we measuring the right outcomes? If novel medicines can effectively lead to turning severe haemophilia into mild haemophilia – will they undermine the importance of comprehensive care? Along with better efficacy, do they bring risks?

As with every change, moving forward with EHLs, novel non-factor replacement treatments and gene therapy will require, in the first place, education for patients, doctors, policy-makers, pharmaceutical companies and health care workers. To create this network of education, on February 28<sup>th</sup>, the EHC held its first-of-the-year Round Table of Stakeholders on the topic of ‘Economics and Access, Health Care Systems and Novel Therapies’ in the European Parliament. Over 40 participants representing the above-named stakeholders joined the conversation on novel haemophilia treatments and the changing landscape they are bringing on a political and economic level. Chairing the event, Member of the European Parliament (MEP) Norica Nicolai was joined by her colleagues MEPs Dr Miroslav Mikolášik and Dr Cristian Buşoi in support of the rare bleeding disorders community and to provide

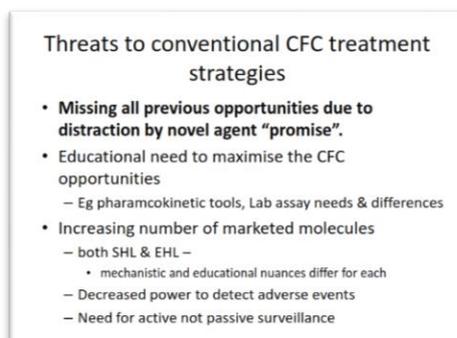
input on what political action needs to be taken to effectively steward the research, regulatory and policymaking landscape through this next phase of haemophilia treatment.

*“With novel therapies, we need to work together to make sure they don’t come on top of inequalities but rather are received in a timely manner all over Europe.”*

*MEP Miroslav Mikolášik*

Economic theory defines rational behaviour as a decision-making process which is based on making choices that result in the most optimal level of benefits or utility for the individual. So how will novel therapies be economically valued and will the balance between price and patient benefit be weighted in in this estimation? The introduction of extended half-life (EHL) products has already changed the way we think about treating patients, mainly from the angle of achieving the same trough levels as with standard treatment products but with less number of infusions. Less infusions could mean less economic burden on the health care system, especially in the long-term.

The differences in outcomes pile up even more when looking at novel treatments and gene therapy. As speakers at the Round Table went through this progression of change (EHL → novel treatments → gene therapy), it was stressed that looking solely at price doesn’t accurately portray economic value. With novel therapies, we are in a situation where a patient can move from a life with spontaneous bleeds to a spontaneous life, not having to plan around when their treatment will take place. This in itself is an economic factor and a value that should be part of the equation.



Slide from the presentation of Dr Dan Hart, member of the EHC Medical and Scientific Advisory Board, on ‘Opportunities and Threats of Novel Agents’.

It is also important to acknowledge that while discussions around new therapies are crucial, we must not miss the opportunities that the treatments we now have in front of us hold. Current standard of care has brought along increased affordability, availability, access and personalisation of treatment, which was the wish list just a short amount of time ago. Being distracted with what will happen in the years to come threatens optimising these opportunities that have translated from a wish list to reality.

Other questions worth thinking about are whether the haemophilia community is prepared to switch to novel therapies and whether comprehensive care has a future, as novel therapies hold the potential to “downgrade” people with severe haemophilia to the mild form of the condition. And though we are excited to be in a time where we are able to ask these questions, we have to pause and think about how to pave the way for new treatments and how the health care system will adapt to them.

As covered during the Round Table, regulation and legislation policies are working to integrate all these questions early on. But policy is not just about content or competence, but also about timing. Given the strong political support assured by the attending Members of the European Parliament and the review of several relevant legislations by the European Commission, there is little doubt that novel therapies will politically find a home.