AISBL European Haemophilia Consortium (EHC) No. 887.106.966
Rue de l'Industrie/ Nijverheidsstraat 10, 1000 Brussels, Belgium
Tel. +32 (0) 2 894 23 70; Email: office@ehc.eu; Website: www.ehc.eu

Event report: EHC Round Table of Stakeholders on "Economics and Access, Health Care Systems and Novel Therapies"

About the event

On Tuesday 27 February 2018, the European Haemophilia Consortium (EHC) held its first Round Table of the year on at the European Parliament in Brussels to discuss the critical issues around novel therapies and their consequences in the dawn of new haemophilia products coming on to the market. The Round Table was hosted by Member of the European Parliament (MEP) Norica Nicolai (ALDE, Romania). MEP Dr Miroslav Mikolášik (EPP, Slovakia) and MEP Dr Cristian-Silviu Buşoi (EPP, Romania) also took part in the discussion with key interventions on the topic. The three MEPs used the opportunity to reiterate their political support to the rare bleeding disorders community.

Over fifty participants attended the event, including patient representatives, industry representatives, health care professionals, academics and policy makers. The event's agenda, list of participants and speakers' presentations can be consulted online on the EHC website. Pictures from the event can be found here.

About novel therapies

Until recently the main haemophilia treatments have been coagulation factor VIII (FVIII) and factor IX (FIX) replacement concentrates. Recently however, longer lasting FVIII and FIX therapies, known as extended half-life (EHL) therapies, have come onto the market. Other novel treatment approaches, such as non-replacement therapies and gene therapies, are currently being developed. With the increasing availability of EHL factor concentrates in Europe and with the probable future licensing of new non-factor concentrate options for people with haemophilia, there is excitement and hope in the patient community around the promising potential to decrease infusion frequencies, increase quality of life and offer alternatives for inhibitor patients. Accordingly, the European market is expected to change significantly. However, there are pending questions on the challenges faced by patients and clinicians not only in obtaining these treatments, but also on their clinical opportunities and possible risks.

The EHC, together with their dedicated supporters from the European Parliament Rare Bleeding Disorders Group, are committed to ensuring that haemophilia patients have the broadest pallet of treatment options available across Europe, and that these are fully understood by all key stakeholders.

Findings and discussions

EHL products, non-factor replacement therapies and gene therapies are on the horizon and will likely bring disruptive change not only to treatment regimens and patients' quality of life, but also to health care systems. To ensure the best and most efficacious use of new treatments, before switching from one product to another, it is recommended to consider the pharmacokinetic (PK) guidance, where possible and relevant, and the specifics of each individual patient. This will significantly enhance the impact of new treatments and maximize protection against bleeds, while also ensuring the right cost-benefit relationship for health care systems. An appropriate balance will also need to be found between benefits and risks for each patient. Other factors, including the cost for the health care system, will also be key considerations when switching patients from one product to another. While full of promise, many novel therapies also

bring completely new modes of action, which will need to be thoroughly understood by health care providers and patients alike. For this reason, there is an urgent need for education of all stakeholders.

Novel therapies are likely to have other impacts as well. By moving patients from severe to moderate or mild forms of the disorder, it will be critical to work towards maintaining the comprehensive care structures established over many years in Europe. Potentially patients may also begin to see non-haematology specialists. It will therefore be paramount to understand the impact of novel therapies on the full health care provision system for the patient and to work towards ensuring that this system continues to exist and meets patient needs in the future.

There are opportunities for conventional Clotting Factor Concentrate (CFC) treatment strategies after the introduction of novel therapies, as well. Many European countries may initiate treatment for some or all of their haemophilia patients with new generations of products, which will lead to lower annual costs for the treatment of patients with haemophilia. This can in itself lead to increased affordability, increased availability, personalisation and increased bleed protection with CFC treatment in many European countries. Ultimately, the vision of treatment is for the co-existence of multiple treatment options for people with haemophilia A and B, which leads to a better quality of life.

In order to increase such options for patients, the European Medicines Agency (EMA) offers support to pharmaceutical companies through various mechanisms, such as the PRIME scheme, accelerated assessment, scientific advice, post-authorisation safety and efficacy follow-up and Advanced Therapeutic Medicinal Products (ATMPs) certification. The EMA has also published several guidelines in the field – e.g. reflection paper on management of clinical risks deriving from insertional mutagenesis and the guidelines on safety and efficacy follow-up and risk management of ATMPs.

Clearly the current market landscape is changing. The increased competition between current and novel therapies brings opportunities for all, in particular patients, but also raises the need for proper education by all members of the health care system. MEPs reiterated their support to patients to ensure that at European level, the right actions are taken to help ensure patient access and choice. Several legal public health provisions exist, including the Orphan Medicinal Products Regulation, the Cross-Border Healthcare Directive, the Medical Devices Regulation and the Advanced Therapeutic Medicinal Products Regulation. With the blood, tissues and cells legislation currently under review, the Health Technology Assessment (HTA) proposal for a Regulation on the table and the OMP/Paediatric Regulation on-going joint assessment, it seems that European provisions for novel therapies are no longer a utopian concept.

Conclusions

With novel therapies coming to market, every stakeholder must work together to ensure that novel, as well as current, treatments are made available and received in a timely manner by all patients across Europe. From a specifically European Union (EU) perspective, ensuring timely access to novel treatments can be delicate due to the EU subsidiarity principle.[1] However, as noted often by MEPs and underscored again by MEP Norica Nicolai during the event, for rare diseases there is a need for a common and standard European approach. Thus, the European Parliament Rare Bleeding Disorders Group reiterated its support to make patient voices heard and their positions reflected in upcoming European legislation, such as for example the HTA proposal for a Regulation being released by the European Commission. The EHC will remain in close contact with their MEP supporters and work together with them to advance patients' needs on these and all issues.

[1] *In areas in which the European Union does not have exclusive competence (e.g. health), the principle of subsidiarity, laid down in the Treaty on European Union, defines the circumstances in which it is preferable for action to be taken by the Union, rather than the Member States*. Available at: http://www.europarl.europa.eu/atyourservice/en/displayFtu.html?ftuId=FTU_1.2.2.html