Addressing the needs of individuals with haemophilia A or von Willebrand disease

ECTH 2018
Marseille, France
Thursday 25th October 2018
12:15 – 13:15
Location: Room Endoume 1

This scientific symposium is for healthcare professionals only

octapharma

Addressing the needs of individuals with haemophilia A or von Willebrand disease

Individuals with haemophilia A (HA) or von Willebrand disease (VWD) want convenient therapies that combine excellent bleeding protection with good tolerability and low immunogenicity.

In haemophilia A, prophylactic replacement of deficient endogenous FVIII is a well-proven physiological approach to therapy. Personalised prophylaxis allows patient care to be adapted according to an individual's needs and lifestyle and helps avoiding over- or under-treatment. The recent NuPreviq study with simoctocog alfa demonstrated that pharmacokinetic (PK)-guided personalised prophylaxis provided excellent bleeding protection and enabled the dosing interval to be extended to twice weekly or less frequently in over half of the patients. Population-based approaches to personalising treatment with simoctocog alfa have been developed, which require only 2 to 4 blood samples to predict an individual's PK profile.

The risk of inhibitor development remains the most significant concern in previously untreated patients with haemophilia A. New insights into risk factors for inhibitor development have emerged from an analysis of the ongoing NuProtect study.

Octapharma is developing a new product with the aim of providing a recombinant FVIII therapy that can be delivered subcutaneously. This method of administration may provide a more convenient treatment option for patients and caregivers and avoid the complications associated with the need for venous access. This could help improve adherence and facilitate the initiation of prophylaxis as early as possible in a patient's life.

VWD is a paradoxical and frustrating disease, and managing people with VWD can be a real challenge. Real-world experience with a high-purity 1:1 VWF/FVIII concentrate will be presented. The physiological ratio of VWF to FVIII simplifies dosing and monitoring.

Both simoctocog alfa and a 1:1 VWF/FVIII concentrate provide 'natural' solutions for individuals with haemophilia A or VWD. The use of these products continues to evolve to meet the changing needs of patients.

DISCUSSION AGENDA

TIME	SPEAKER	DISCUSSION TITLE
12:15	**Yesim Dargaud**	Welcome and introduction by the Chair
12:20	**Carmen Escuriola Ettingshausen**	Going further with simoctocog alfa: Addressing the individual needs of people with haemophilia A
12:40	**Christoph Kannicht**	Taking FVIII into the future: The development of a subcutaneous recombinant human FVIII for the treatment of haemophilia A
12:55	**Erik Berntorp**	Real-world experience with a 1:1 VWF/FVIII concentrate in patients with von Willebrand disease
13:10	**Yesim Dargaud**	Discussion and Conclusions