European study of real-life haemophilia treatment emphasizes need to improve standard of care

Swedish Orphan Biotech AB (publ) (Sobi) (STO: SOBI) announces the results from a new European study that assessed the efficacy of haemophilia care in real life. The study, which was fully funded by Sobi, showed that treatment practice varied widely between countries and that patients treated on-demand and prophylactically both experienced bleeds, emphasizing the need for further enhancing standard of care.

The authors concluded that prophylaxis should be considered for people with frequent bleeds to avoid pain and deterioration of joint function, and the treatment regimen for people on prophylaxis needs improvement.

The study has been published in the on-line issue of Haemophilia, the official journal of the World Federation of Haemophilia.[i]

“The overall results indicate that there is a significant need to advance standard of care within haemophilia”, said Stefan Lethagen, Vice President Medical & Clinical Sciences, Haemophilia at Sobi. “Even when prophylaxis is the norm, it appears that prophylactic treatment is driven to the minimal acceptable level or even lower, which increases the risk of joint injury and limits the ability to live a full and active life.”

The retrospective study included 1346 people with haemophilia A and 312 people with haemophilia B, from seven European countries. The study was designed to provide insights into current haemophilia treatment practice and outcome.

Prophylaxis was overall found to be the most dominating treatment for people with severe haemophilia A. It was the most common treatment regimen among children and decreased with increasing age. On-demand treatment was reported to be most common in moderate haemophilia A, and there was no trend across age groups. For people with haemophilia B, prophylaxis was most common in four out of seven countries.

In this European study, the median ABR for patients with severe haemophilia A on prophylaxis was as high as four in some countries and some patients experienced more than 12 bleeds per year, likely reflecting insufficient therapy, inappropriate dose-interval, presence of target joints, poor adherence or difficulty of correctly assessing bleeds by some patients. The authors emphasised that there is also a need for improvement in individuals with moderate haemophilia A on prophylaxis, since bleeds were as frequent or more frequent than for those with severe disease on prophylaxis. The overall results of the study indicate that there is room for improvement of haemophilia therapy, even for patients currently on prophylactic treatment.

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About Haemophilia A and B

Haemophilia is a rare, genetic disorder in which the ability of a person's blood to clot is impaired. Haemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. Haemophilia B occurs in about one in 25,000 male births annually, and more rarely in females. Worldwide, it is estimated that more than 400,000 people are living with haemophilia.

People with haemophilia A or B experience prolonged bleeding episodes that can cause pain, irreversible joint damage and life-threatening haemorrhages. Prophylactic infusions of factor VIII or IX can temporarily replace the missing clotting factors that are needed to control bleeding and prevent new bleeding episodes.[ii] The World Federation of Hemophilia recommends prophylaxis as the optimal therapy as it can prevent bleedings and joint destruction.[ii]

About Sobi

Sobi is an international specialty healthcare company dedicated to rare diseases. Sobi’s mission is to develop and deliver innovative therapies and services to improve the lives of patients. The product portfolio is primarily focused on Haemophilia, Inflammation and Genetic diseases. Sobi also markets a portfolio of specialty and rare disease products across Europe, the Middle East, North Africa and Russia for partner companies. Sobi is a pioneer in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2015, Sobi had total revenues of SEK 3.2 billion (USD 385 M) and about 700 employees. The share (STO: SOBI) is listed on Nasdaq Stockholm. More information is available at www.sobi.com

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Stefan Lethagen, Vice President Medical & Clinical Sciences, Haemophilia at Sobi. The overall results indicate that there is a significant need to advance standard of care within haemophilia