Advances in Haemophilia:

Factor-Based Therapies and Long-Term Evidence Versus New Treatment Modalities

Monday 21 May 2018, 18:15–19:45
Hall 3, Scottish Event Campus (SEC), Glasgow, UK

Satellite Symposium Hosted by Sobi™ (Swedish Orphan Biovitrum AB (publ)) and Bioverativ, a Sanofi company

WFH 2018 World Congress

Chair

K. John Pasi
UK

Dear Colleague,

On behalf of Sobi™ (Swedish Orphan Biovitrum AB (publ)) and Bioverativ, a Sanofi company, I would like to invite you to the Satellite Symposium which will take place on **Monday 21 May 2018 in Hall 3, Scottish Event Campus (SEC), Glasgow, UK, from 18:15–19:45.**

I look forward to seeing you at this engaging and informative scientific symposium.

With kind regards,

K. John Pasi (Chair)

Professor, MD, PhD
Barts and the London School of Medicine and Dentistry
London, UK

Agenda

18:15 – 18:20	**Welcome and Introduction**	Chair: K. John Pasi, UK
18:20 – 18:35	**Walking a Tightrope: Understanding and Handling a Defective Coagulation Cascade**	Peter Lenting, France
18:35 – 18:50	**Inhibitor Management: Making the Appropriate Clinical Choice for Patients in Critical Situations**	K. John Pasi, UK
18:50 – 19:10	**Joint Disease - Still a Hallmark of Haemophilia Today?**	Johannes Oldenburg, Germany
19:10 – 19:30	**Fc-Fusion Factors: Long-Term Data**	Roshni Kulkarni, USA
19:30 – 19:45	**Questions & Answers**	Faculty
	Closing Remarks	K. John Pasi, UK

Access to this symposium will be restricted to healthcare professionals only.
Non-healthcare attendees will not be granted access.

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▼ Elocta® (Please refer to the EMA website or UK API)
▼ Alprolix® (Please refer to the EMA website or UK API)
▼ This medicinal product is subject to additional monitoring

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ELOCTA®▼ 250 IU, ELOCTA® 500 IU, ELOCTA® 1000 IU, ELOCTA® 1500 IU, ELOCTA® 2000 IU and ELOCTA® 3000 IU powder and solvent for solution for injection.

Abbreviated Prescribing Information for UK/Ireland.

▼ This medicinal product is subject to additional monitoring

Refer to the appropriate Summary of Product Characteristics (SmPC) before prescribing ELOCTA®.

ELOCTA®: Contains efmoroctocog alfa, respectively at 250 IU (83 IU/mL); 500 IU (167 IU/mL); 1000 IU (333 IU/mL); 1500 IU (500 IU/mL); 2000 IU (667 IU/mL); 3000 IU (1000 IU/mL). Also contains 14 mg equivalent to 0.6 mmol of sodium per vial. **Indication:** Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency). ELOCTA® can be used for all age groups. **Dosage and Administration:** Intravenous use. Requires supervision by a physician experienced in haemophilia treatment. One IU of efmoroctocog alfa is equivalent to one IU of factor VIII in a millilitre of normal human plasma. The rate of administration should not exceed 10 mL/min. Treatment dose and duration depend on the severity of factor VIII deficiency, the location and extent of bleeding, and the patient's clinical condition. Dose guide: *Prophylaxis:* The recommended dose is 50 IU/kg every 3 to 5 days. The dose may be adjusted based on patient response in the range of 25 to 65 IU/kg. In some cases, especially in younger patients, shorter dosage intervals or higher doses may be necessary. *On demand:* For ELOCTA® dosing in the treatment of bleeding episodes and surgery, refer to the SmPC. The amount to be administered and the frequency of administration should always be oriented to the clinical effectiveness in the individual case (see section 5.2 in the SmPC). The time to peak activity is not expected to be delayed. **Contraindications:** Hypersensitivity to efmoroctocog alfa or other ingredients. **Warning and Precautions:** Allergic hypersensitivity reactions are possible. Ensure patients are familiar with the signs of hypersensitivity. Discontinue use if reactions occur. Carefully monitor for the development of neutralising antibodies (inhibitors) to factor VIII. In cases of high inhibitor levels, consider other therapeutic options. May increase cardiovascular risk in patients with existing cardiovascular risk factors. Consider risks associated with use of central venous access device complications, if appropriate. It is recommended that the name and batch number of the product are recorded. Contains sodium. **Interactions:** None known. However, in the absence of compatibility studies, do not mix this medicine with other medicinal products. **Undesirable Effects:** Hypersensitivity or allergic reactions. For a full list of side effects, refer to the SmPC. **Legal Category:** Medicinal product subject to restricted medical prescription. **Pack size:** 1 unit (glass vial of powder with 3 mL solvent in a glass pre-filled syringe plus materials for reconstitution and infusion), NHS List Price: £ 0.85/IU. Eire List Price: Available on request. **MA Numbers:** EU/1/15/1046/001 – 007. **MA Holder:** Swedish Orphan Biovitrum AB (publ), SE-112 76 Stockholm, Sweden. **Local representative:** Sobi Ltd Suite 2, Riverside 3, Granta Park, Great Abington, Cambridgeshire, CB21 6AD. Additional information and full Prescribing Information is available on request from the local representative. **Date of Preparation:** June 2016. PP-1300.

Prescribing information may differ between countries

Adverse events should be reported. For reporting within the UK, forms and information can be found at www.mhra.gov.uk/yellowcard and for Republic of Ireland at www.hpra.ie. Adverse events should also be reported to Swedish Orphan Biovitrum Ltd at drugsafety@sobi.com

▼ ALPROLIX® (eftrenonacog alfa)

Abbreviated Prescribing Information

For further prescribing information, please refer to the ALPROLIX® Summary of Product Characteristics (SPC)

Composition: The active substance is eftrenonacog alfa (recombinant coagulation factor IX, Fc fusion protein). Each vial of ALPROLIX® contains nominally 250, 500, 1000, 2000 or 3000 IU eftrenonacog alfa. The other ingredients are sucrose, L-histidine, mannitol, polysorbate 20, sodium hydroxide and hydrochloric acid. **Diluent:** Sodium chloride and water for injection. **Indications:** Indicated for the treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency). ALPROLIX® can be used for all age groups. **Dosage and Administration:** *On-demand treatment:* The calculation of the required dose of recombinant factor IX Fc is based on the empirical finding that 1 International Unit (IU) factor IX per kg body weight raises the plasma factor IX activity by 1% of normal activity (IU/dL). The required dose is determined using the following formula: Required units = body weight (kg) x desired factor IX rise (%) (IU/dL) x {reciprocal of observed recovery (IU/kg per IU/dL)}. Please refer to the SmPC for further information, including Table 1: Guide to ALPROLIX® dosing for treatment of bleeding episodes and surgery. *Prophylaxis:* For long-term prophylaxis, the recommended dose is *either* 50 IU/kg once weekly, dose adjusted based on individual response, *or* 100 IU/kg once every 10 days, interval adjusted based on individual response. Some patients who are well-controlled on a once every 10 days regimen, might be treated on an interval of 14 days or longer. In some cases, especially in younger patients, shorter dosage intervals or higher doses may be necessary. The highest recommended dose for prophylaxis is 100 IU/kg. *Treatment monitoring:* Please refer to the SmPC for further information on treatment monitoring. *Elderly population:* There is limited experience in patients ≥65 years old. *Previously untreated patients (PUPs):* The safety and efficacy of ALPROLIX® in PUPs have not yet been established. *Paediatric population:* For children <12 years old, more frequent or higher doses may be required and the recommended starting dose is 50-60 IU/kg every 7 days. For adolescents (≥12 years old), the dose recommendations are the same as for adults. Refer to the SmPC for instructions on reconstitution. **Contraindications:** Hypersensitivity to eftrenonacog alfa (recombinant human coagulation factor IX, and/or Fc domain) or to any of the excipients. **Precautions and Warnings:** Allergic type hypersensitivity reactions are possible. Patients should be informed of the signs of hypersensitivity reactions. Patients should be advised to discontinue use of the product immediately and contact their physician if such signs occur. Implement standard treatment in cases of anaphylactic shock. All patients treated with coagulation factor IX products should be carefully monitored for the development of inhibitors. Patients with liver disease, postoperative patients, newborn infants, and patients at risk of thrombotic phenomena or coagulopathy should be monitored for early signs of thrombotic complications. In patients with existing cardiovascular risk factors, substitution therapy with factor IX may increase the cardiovascular risk. If a central venous access device (CVAD) is required, risk of CVAD-related complications including local infections, bacteraemia and catheter site thrombosis should be considered. Recording of batch number is recommended in order to maintain a link between the patient and the batch of the medicinal product. The listed warnings and precautions apply both to adults and children. ALPROLIX® contains 0.3 mmol (6.4 mg) sodium per vial. This should be taken into consideration by patients on a controlled sodium diet. **Interactions:** No interactions of human coagulation factor IX (rDNA) with other medicinal products have been reported. No interaction studies with ALPROLIX® have been performed. **Undesirable Effects:** Hypersensitivity or allergic reactions (swelling of the face, rash, hives, tightness of the chest and difficulty breathing, burning and stinging at the infusion site, chills, flushing, generalised urticaria, headache, hypotension, lethargy, nausea, restlessness, tachycardia, vomiting, wheezing) have been observed rarely and may in some cases progress to severe anaphylaxis (including shock). Nephrotic syndrome has been reported following attempted immune tolerance induction in haemophilia B patients with factor IX inhibitors and a history of allergic reaction. In post-marketing experience, FIX inhibitor development and hypersensitivity have been observed. Patients with haemophilia B may develop neutralising antibodies (inhibitors) to factor IX. The use of low purity factor IX products has been associated with instances of myocardial infarction, disseminated intravascular coagulation, venous thrombosis and pulmonary embolism. The use of high purity factor IX is rarely associated with thromboembolic complications. Consult the SmPC for further information about adverse events. **Legal Category:** POM **Marketing Authorisation Nos.:** EU/5/16/1098/001-005. **Pack size:** 1 glass vial of powder plus materials for reconstitution and infusion. NHS List Price: £1.20/IU. Eire List Price: Available on request. **Marketing Authorisation Holder:** Swedish Orphan Biovitrum AB (publ), SE-112 76 Stockholm, Sweden.**Further information** is available from Swedish Orphan Biovitrum (UK) Ltd, Suite 2, Riverside 3, Granta Park, Great Abington, Cambridgeshire, CB21 6AD **Date of Revision:** June 2017 **Reference:** PP-2703

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