Documents

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Ultra-Long factor VIII: a major step forward toward a hemophilia-free mind
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Abstract
A remarkable step forward in the treatment of hemophilia A has recently been achieved with the development of an Ultra-Long modified factor (F)VIII. Leveraging expertise gained with fusion to immunoglobulin Fc fragments, disconnecting FVIII from endogenous von Willebrand factor (via a D′-D3 fragment), and benefiting from the pharmacokinetic prolongation provided by the addition of hydrophilic polypeptides, efanesoctocog alfa opens a new era in the treatment of hemophilia A. The term Ultra-Long FVIII has been proposed to designate it and differentiate it from extended half-life FVIII. The level of FVIII correction within the normal range for several days provided by this molecule should allow an increasing number of patients to free themselves from the physical and psychological constraints of hemophilia A. Certainly, the burden of weekly intravenous infusions persists but is compensated by a correction of hemostasis whose amplitude and duration remain unmatched by other therapeutic options currently available. © 2024 International Society on Thrombosis and Haemostasis

Author Keywords
extended half-life; factor VIII concentrate; hemophilia; innovation; normalization

Index Keywords
blood clotting factor 8, blood clotting factor 8 concentrate, blood clotting factor 9, immunoglobulin Fc fragment, polypeptide, recombinant blood clotting factor 8, von Willebrand factor, blood clotting factor 8, coagulating agent, F8 protein, human, fusion protein; clinical observation, controlled study, diffusion of innovation, drug therapy, duration, genotype, half life time, hemarthrosis, hemophilia, hemophilia A, hemophilia B, hemostasis, human, intravenous drug administration, metrorrhagia, muscle injury, pharmacokinetics, phenotype, quality of life, Review, thrombosis, animal, blood, blood clotting, drug effect, half life time, hemophilia A, metabolism, treatment outcome; Animals, Blood Coagulation, Coagulants, Factor VIII, Half-Life, Hemophilia A, Hemostasis, Humans, Immunoglobulin Fc Fragments, Infusions, Intravenous, Recombinant Fusion Proteins, Treatment Outcome, von Willebrand Factor

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