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Newer standardized and cost effective management approaches for prophylaxis of pediatric hemophiliacs with recombinants and long-acting FVIII & FIX drugs using chromogenic assays: A concise review

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Prophylaxis of hemophilia, identified early in life, is becoming an important requirement from affected patients and society, in all countries demanding treatments for managing hemorrhages. Such a therapeutic modality highly improves hemophiliacs' quality of life, through a dramatic reduction of severe bleeding events and disease burdens. Availability of recombinant proteins has introduced an abundant offer of therapeutic products and overwhelms limitations of blood extracted products. The recent introduction of long-acting products reduces the frequency and constraints of prophylaxis therapy, but there is an important cost for public health and individual monitoring of drug kinetics in treated patients allows optimizing the associated costs, as conventional clotting assays are APTT based methods, prerequisite FVIII or FIX deficient. New recombinant and long-acting products (Glyco-PEGylated, Fc or human albumin fusion proteins) can present huge differences with the different APTT reagents and drug-specific calibrators can be needed. Availability of multiple drugs, which present different behaviors in the assays, renders APTT based clotting assays inappropriate for monitoring drug kinetics in treated patients. Chromogenic assays are now available for overcoming these inconveniences: All blood extracted, recombinant or long-acting products generate the same dose-response curves for the same product potency. We have experience with Biophen™ Factor VIII and Biophen™ Factor IX chromogenic assays, which can be calibrated using plasma calibrators with assigned FVIII or FIX concentrations, and can be used for all drugs. Dynamic ranges cover all concentrations from <10% to >200% FVIII or FIX activity, and low range protocols are available for concentrations down to 0.1%. Assays are automatable on all coagulation platforms, and can be used with the micro-ELISA plate format for high throughput testing. Multicentric studies have demonstrated the robustness of these chromogenic assays with good reproducibility and accuracy, and high reliability between centers. These assays are now widely used for the various FVIII or FIX drug potency value assignment in pharmaceutics industry. Internal standards are available. They are established against the WHO International Standards, for FVIII or FIX concentrates, and they are prepared and supplied by NIBSC (Potters Bar, UK).

Biography

Jean Amiral has been working for 40 years in the coagulation area, first as Scientific Director of Diagnostica Stago (until 1998), then as the Founder of Hyphen BioMed as its Scientific and Research Director (until 2012), and is now Scientific and Technical Consultant for Hyphen BioMed and Sysmex. He has developed many assays for hemostasis and thrombosis and participated in many studies.

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