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G. Sottilotta**NEW THERAPEUTIC HORIZONS IN HEMOPHILIA B: HOW TO GET MORE PROTECTION FOR PATIENTS**

What is the rationale for a new concentrate of a Factor IX (FIX) Extended Half Life? The reduction of the Annual Bleeding Ratio (ABR) and the prevention of further hemarthrosis in the target joints through weekly infusions. Other objectives are: the improvement of the joint status and the quality of life thanks to the possibility of reducing the frequency of prophylactic infusions, and the reduction of the risk of inhibitors. Nonacog beta pegol N9-GP (Refixia, Novonordisk) is an increased half-life recombinant FIX (rFIX) that meets all these needs. Refixia is produced in Chinese hamster ovary (CHO) cells by a serum-free manufacturing process. What are its characteristics? Refixia is a purified rFIX) with a 40 kilo Dalton (kDa) polyethylene-glycol (PEG) selectively attached to specific N-linked glycans in the rFIX activation peptide. Once activated, the activation peptide comprising the 40 kDa polyethylene glycol fraction separates, leaving the

native factor IX molecule (identical to human FIX) activated. In Europe, Refixia is approved for the treatment and prophylaxis of bleeding in patients aged 12 years and above with haemophilia B. The market placing was preceded by several years of clinical studies. The completed clinical trial program (Paradigm) included one phase 1 trial and four phase 3 multicenter, noncontrolled trials.

All clinical studies have given excellent results in terms of efficacy and safety compared to the other factor IX concentrates already on the market with which they were compared. In a dedicated surgery trial, the haemostatic effect of Refixia during surgery was confirmed with a 100% success rate in the 15 major surgeries in the trials. All minor surgeries evaluated were performed successfully.

Two real-life Canadian studies also found that overall, all patients studied reported favorable experiences after switching to Refixia prophylaxis and a reduced need for the number of infusions for those treated on-demand. The authors conclude that the extended half-life of N9-GP is likely to contribute to improvements in the quality of life.

