Management of von Willebrand disease with a factor VIII-poor von Willebrand factor concentrate: Results from a prospective observational post-marketing study

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ARTICLE

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Abstract

Background

A triple-secured plasma-derived von Willebrand factor (pdVWF) almost devoid of factor VIII (FVIII):WILFACTIN^® , was approved in France in 2003, and then in other countries for the treatment of patients with von Willebrand disease (VWD).

Objective

To investigate long-term safety and efficacy of the product in real-life over the first 5 post-approval years.

Patients/Methods

This prospective, observational, national post-marketing study (PMS) enrolled patients of all ages and VWD types. Patients were observed for up to 3 years and treated for one or more occasions. Efficacy was assessed for each major event. Breakthrough bleeding rate 3 days post-infusion and annualized bleeding rate (ABR) were also evaluated for long-term prophylaxis.

Results

Overall, 155 of 174 patients enrolled from 31 centers were eligible for efficacy assessment. Most patients (76.8%) were severely affected (VWF:RCo ≤ 15 IU/dL). They were treated for 743 bleeds and 140 surgeries including childbirth. Efficacy outcomes were excellent/good for 98.2% of 56 major surgeries and 94.0% of 67 major bleeds. Approximately 75% of 49 major mucosal bleeds were effectively managed without FVIII co-administration. In 32 patients receiving prophylaxis, breakthrough bleeding occurred in 1.5% of infusions and median ABR was 1.0 for 20 patients treated ≥ 12 months. Excellent tolerability was confirmed with no safety concerns. No thrombotic events were observed.

Conclusions

Results from this PMS increase the clinical experience of a FVIII-poor pdVWF in patients of all ages and VWD types including those with thrombotic risk factors and emphasize that giving FVIII is not always mandatory to effectively treat patients with severe VWD.

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