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A retrospective chart review to assess clinical characteristics of women and girls with factor VIII and IX deficiency

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Abstract

Objective: Evaluate clinical characteristics, hemostasis management, and clinical outcomes regarding menstruation, child birth, surgical procedures, dental care, and spontaneous and traumatic bleeds of women and girls with factor VIII (FVIII; hemophilia A) or factor IX (FIX; hemophilia B) deficiency (WGFD). Methods: A retrospective chart review is ongoing at three US hemophilia treatment centers (HTC) to collect data on WGFD (obligate or potential carriers of FVIII or FIX deficiency, with or without genetic confirmation). Data are collected on patients who had at least two HTC visits and underwent medical or surgical interventions for hemostasis management between April 2012 and November 2018, with the outcome available in medical charts. Summary: Interim results as of April 5, 2019 include charts from two HTCs on 26 (89.7%) patients with FVIII deficiency and 3 (10.3%) patients with FIX deficiency. The median (range) age at factor deficiency diagnosis was 18.5 (0.1–72.0) years. Twenty-four (82.8%) and 8 (27.6%) patients had a family history of hemophilia and other bleeding disorders, respectively. A total of 17 (58.6%) patients initially visited the HTC due to family history/genetic counseling. Other reasons for visiting an HTC were heavy menstrual bleeding (n=12 [41.4%]) or spontaneous or traumatic bleeds (n=12 [41.4%]), including 7 (24.1%) patients reporting both heavy menstrual bleeding and spontaneous or traumatic bleeds. Of the 12 patients with spontaneous or traumatic bleeds, 4 (33.3%) patients had joint bleeds, 6 (50.0%) patients had excessive nose bleeds, and 9 (75.0%) patients had easy bruising. For those with FVIII deficiency, the median (range) FVIII level at diagnosis was 32.5 (2.0–101.1) IU/dL (n=24), median (range) baseline hemoglobin was 12.9 (5.4–14.8) g/dL (n=19), and median (range) baseline von Willebrand factor ristocetin cofactor was 70 (40–150) IU/mL (n=16). The median (range) number of documented bleeds was 1.0 (0.0–24.0) in the first year at the HTC. Final results of this chart review, including data from those with FIX deficiency, HTC interventions, and outcomes for hemostasis management, will be presented. Conclusions: This chart review provides further insights into the clinical presentation and hemostasis management of WGFD evaluated at HTCs in the US. Results may contribute to the design of future prospective studies evaluating treatment options for this patient group.