

## **A Retrospective Study Evaluating Immune Tolerance Induction (ITI) with a Plasma-derived Factor VIII for Patients with Hemophilia A and High Titer Inhibitor**

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### **Submission Group**

Inhibitors

### **Abstract**

**Objective:** The formation of inhibitors to clotting factors is a serious complication in hemophilia A. Immune tolerance induction (ITI) therapy remains the primary method for eradicating inhibitors. This multicenter retrospective data collection project evaluated patient- and treatment-related factors associated with outcomes following primary or rescue ITI with an antihemophilic factor (Human) concentrate in patients with hemophilia A and high titer inhibitors. **Methods:** Medical records of nine inhibitor patients treated with antihemophilic factor (human) for primary or rescue ITI therapy between January 1, 2012 and July 31, 2017 were evaluated in four US hemophilia treatment centers. Data were de-identified and analyzed descriptively. Outcome measures were defined per the International Immune Tolerance Induction Study: successful (eradication of FVIII inhibitor and normal FVIII recovery), partial success (near normal FVIII recovery), and failure. **Results:** A total of nine patients between the ages of 10 months and 39 years at time of ITI were evaluated. Six out of nine patients (66.7%) had successful ITI; three with complete success (ages 27, 32, 32 years) and three with partial success (ages 5, 5, 21 years). Three patients failed ITI (ages 1.5, 10.5, 39 years) (Table 1.) Six of the patients had a combined previous ten attempts at ITI with other products (plasma derived and/or recombinant). Of these six rescue patients, ITI with antihemophilic factor (human) was successful in one and partially successful in three. **Conclusions:** While retrospective data has limitations, real-world evidence demonstrates that ITI with antihemophilic factor (human) concentrate can be successful or partially successful in diverse populations of moderately complex patients with hemophilia A and high titer inhibitor.