

Key Questions for Legislative Report and Cost-Effectiveness Analysis (Deliverables due June/August)

1. How do included drugs compare in their efficacy and long-term effectiveness for alleviating symptoms and stabilizing the disease in patients with hemophilia A/B or Von Willebrand Disease?
2. What are the comparative incidence and severity of harms associated with the use of these drugs?
3. Do the included drugs differ in effectiveness or harms in the following subgroups:
 - a. Different genders, or different racial, age, or socioeconomic groups?
 - b. Patients with co-morbidities?
 - c. Patients taking other commonly prescribed drugs?
 - d. Patients with early vs. established disease
4. What evidence is there on indirect medical and non-medical costs, patient reported outcomes (PROs), and quality of life (QoL) measures for patients with hemophilia A/B or Von Willebrand Disease, and what variables of treatment impact indirect costs, PROs, and QoL?
5. What is the estimated cost-effectiveness (cost per outcome and/or cost per QALY) of these therapies?

Key Questions for Guiding Clinical Research and Developing Evidence-based Guidelines

6. What in-home strategies have been evaluated for appropriate use of hemophilia drugs? (e.g. strategies that assure patients who might self-administer at home are doing so for appropriate reasons and with appropriate doses; strategies on in-home nursing services or periodic home care [e.g. following a surgery])?
7. What is the current evidence on using ultrasound to detect bleeds versus non-bleeds in the hemophilia population?
8. What is the current evidence on actual body weight dosing versus ideal body weight dosing versus adjusted body weight dosing for both treatment and prophylaxis in the hemophilia population?

Inclusion criteria

Population

- Adult outpatients with hemophilia A/B or Von Willebrand disease
- Pediatric outpatients with hemophilia A/B Von Willebrand disease

Interventions

Drug Name	Type	Brand Names
Antihemophilic Factor [Factor VIII]	Human	Hemofil M Koate-DVI Monoclate-P
	Recombinant	Eloctate Helixate FS Kogenate FS Novoeight Nuwiq Recombinate Refacto
	Recombinant Porcine	Obizur
Antihemophilic Factor RAHF-PFM [Factor VIII]	Recombinant	Advate
Antihemophilic Factor PAF [Factor VIII]	Recombinant	Xyntha
Antihemophilic Factor/Von Willebrand Factor Complex	Human	Alphanate Humate-P Wilate
Factor IX	Human	AlphaNine SD Mononine
	Recombinant	Alprolix BeneFIX Ixinity Rixubis
3-factor Prothrombin Complex Concentrate [PCC]	Human	Bebulin Bebulin VH Profilnine Profilnine SD
4-factor Prothrombin Complex Concentrate [PCC]	Human	Kcentra
Activated Prothrombin Complex Concentrate [aPCC]	Human	FEIBA
Factor VIIa	Recombinant	NovoSeven RT
Von Willebrand Factor	Recombinant	Vonvendi (FDA Approved Dec 2015)