

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/C 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 clinical strategies have been evaluated with respect to assuring the appropriate use of these drugs? (e.g. strategies that assure patients who might self-administer at home are doing so for appropriate reasons and with appropriate doses)?
5. 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/C or Von Willebrand Disease, and what variables of treatment impact indirect costs, PROs, and QoL?
6. 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

7. What clinical strategies have been evaluated with respect to assuring the appropriate use of these drugs? (e.g. strategies that assure patients who might self-administer at home are doing so for appropriate reasons and with appropriate doses)?
8. Where is the place in therapy for new products in the pipeline currently undergoing clinical trials?
9. What is the current evidence on using ultrasounds to detect bleeds in the hemophilia population?
10. What is the current evidence on actual body weight dosing versus ideal body weight dosing?
11. What is the current evidence on in-home nursing services or periodic home care (e.g. following a surgery) for hemophilia patients?
12. What is the current evidence on using antifibrinolytics or other therapies in addition to clotting factors versus clotting factors alone?

Inclusion criteria

Population

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

Interventions

Antihemophilic Factor (Human)
Antihemophilic Factor (Porcine)
Antihemophilic Factor (Recombinant)
Antihemophilic Factor (Recombinant/Porcine)
Antihemophilic Factor RAHF-PFM
Antihemophilic Factor PAF
Antihemophilic Factor/Von-Willebrand Factor Complex (Human)
Factor IX Complex
Factor XIII Complex (Human)