Key Questions

- 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 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 PROs and QoL?
- 6. What is the estimated cost-effectiveness (cost per outcome and/or cost per QALY) of these therapies?

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)