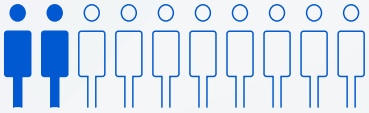
Treatment Burden and Venous Health Complications in People with Hemophilia B without Inhibitors

The Burden of IV Prophylactic Treatment Storage, Preparation and Administration

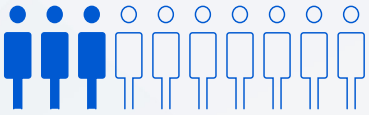
People with hemophilia B without inhibitors request treatments that are **longer-acting, easier to administer** (e.g., subcutaneous), **more manageable** (e.g., less volume to inject)[1] (n=166)

2 in 10 people



with severe hemophilia B without inhibitors on **SHL treatments** report being inconvenienced by medication storage, preparation, and administration[2] (n=9)

>3 in 10 people



with severe hemophilia B without inhibitors on **EHL treatments** report being inconvenienced by medication preparation, and administration[2] (n=36)

Storing medication and supplies is a major burden for patients and caregiver

24% of people with severe hemophilia B without inhibitors on EHL treatments are concerned about storing medication[2] (n=36)

12% of people with hemophilia B without inhibitors are inconvenienced by storing medication[3] (n=112)

15% of people with hemophilia B without inhibitors are inconvenienced by carrying medication and supplies while outside the house[3] (n=112)

Preparing and administering IV prophylactic treatment is a time-consuming and complex process

DID YOU KNOW?

A patient must complete **30 STEPS** to prepare and self-administer IV prophylactic therapy according to Hemophilia of Georgia[4]

Among people with hemophilia B without inhibitors[2]:

20% **on SHL IV prophylactic treatment** (n=9)

14% **on EHL IV prophylactic treatment** (n=36)

are troubled by the number of steps required to prepare and administer IV prophylactic treatment (n=46)

18% have trouble finding time to self-inject[3] (n=112)

The Acute Physical Burden of Intravenous Prophylactic Treatment

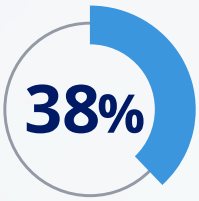
IV prophylactic therapy disrupts patients' lives due to the physical toll of frequent injections, impacting **treatment adherence, clinical outcomes, and physical activities** for people with hemophilia B without inhibitors.

Among patients with hemophilia B without inhibitors[3] (n=112)

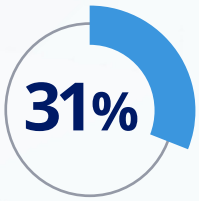
2 in 10 report **soreness** due to injections[3]

>2.5 in 10 experience **pain** due to injections[3]

Among people with hemophilia B without inhibitors,[3]



experience **bruising**[3]



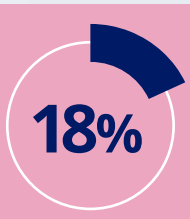
experience **physical discomfort**[3]

Physicians report that (n=81)

100% adherence to their prophylactic treatment regimen only takes place with[5]

of people with hemophilia B **on SHL treatment**

of people with hemophilia B **on EHL treatment**



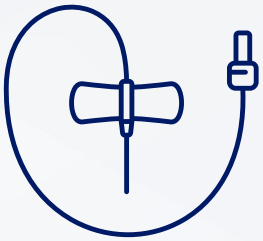
postpone, delay, or miss treatment on purpose[3] (n=112)

Frequent IV prophylactic injections impair long-term venous health for patients with hemophilia without inhibitors

Children and adults experience significant long-term venous health complications due to **scarring, infections, deep-vein thrombosis, and more.**

Easy and safe treatment in children is hindered by the **scarcity of suitable veins** and further challenged by complications due to **CVAD implantation[6]**

Patients requiring CVADs are at risk of infection:



13–48% of implanted catheters may lead to potentially life-threatening conditions,[6-11] including bacterial endocarditis[12]

32–53% of patients experienced CVAD-related deep vein thrombosis after 1 year[13,14] with the risk of loss of potential access sites in arms[6]

People with hemophilia B without inhibitors experience major complications to venous health[3]: (n=112)

experience ***scarring***[3]

experience ***blown or ruptured veins***[3]

Major long-term treatment complications can arise, including[15]:

- **Blood loss**
- **Blood clots**
- **Bruising**
- **Swelling**
- **Permanent artery or vein weakness**
- **Increased risk of injury**

Abbreviations
CVAD, central venous access device; EHL, extended half-life; IV, intravenous; SHL, standard half-life

References
1. Zhang H, *et al*. Poster presented at the WFH 2022 World Congress; 8–11 May 2022. PP--22. 2. Jiménez-Yuste V, *et al*. Poster presented at 66th ASH 2024 Annual Meeting and Exposition; 7–10 December 2024. 5077. 3. Novo Nordisk Data on File. 2025. 4. Hemophilia of Georgia [online]. Steps to prepare and infuse factor. Available at: https://www.hog.org/handbook/article/3/35/steps-to-prepare-and-infuse-factor. Last accessed May 2025. 5. Thakkar S, *et al*. Poster presented at 65th ASH Annual Meeting and Exposition; 9–12 December 2023. P2616. 6. Castaman G and Linari S. *Expert Rev Hematol*. 2018;11:7:567-576. 7. Liesner R, *et al*. *Br J Haematol.* 1995; 91:1:203-207. 8. Blanchette V, *et al*. *Blood Coagul Fibrinolysis* 1997;8 Suppl 1:S11-S14. 9. Santagostino E, *et al*. *Br J Haematol.* 1998;102:5:1224-1228. 10. Langley A, *et al*. *Haemophilia* 2015;21:4:469-476. 11. Khair K, *et al*. *Haemophilia* 2017;23:4:e276-e281. 12. Hothi D, *et al*. *Haemophilia* 2001;7:5:507-510. 13. Journeycake J, *et al*. *Blood* 2001;98:6:1727-1731. 14. Ettingshausen C, *et al*. *Blood* 2002;99(4):1499-1500.15. UPMC [online] October 1, 2024. Available at: https://www.upmc.com/services/heart-vascular/conditions/vascular-trauma. Last accessed: May 2025.