

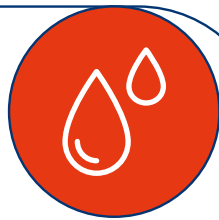
Improving outcomes in hemophilia: identifying unmet needs

Understanding the unmet needs

Outcomes for people with hemophilia have improved, with a wider range of efficacious treatment options and the ability to individualize therapy; however, a range of unresolved treatment and disease burdens remain.¹

Blood protection

- Protection from **muscle and joint bleeds** is an important aspect of hemophilia care¹
- Current management of hemophilia involves **prophylactic factor replacement**, in combination with **on-demand factor administration** for acute bleeding^{1,2}
- Despite prophylactic treatment, some people receiving factor and non-factor therapy with hemophilia **still experience bleeds**³
- Recent real-world data suggest that people with hemophilia **experience anxiety and worry** that their current prophylaxis treatment does not provide **adequate protection** from bleeds⁴
- People living with **hemophilia A and B with inhibitors** experience a **significantly greater burden of bleeding** than those without inhibitors⁵
- In light of **new therapy options**,¹ patients aspire to experience **zero breakthrough bleeds** and a **disease-free mindset**^{6,7,8}
- However, novel therapies are **not always accessible**, with many patients receiving **on-demand or low-dose prophylaxis** that does not fully prevent bleeds⁹



Joint health

- Although prophylactic therapies have improved joint health for people living with hemophilia, they **do not provide complete protection** from target joints^{6,10}
- Recurrent **joint bleeds**, as well as **subclinical bleeding**, can lead to **arthropathy**⁶



By the age of 18 years, **25% of people with hemophilia develop joint damage despite early prophylaxis**¹¹

- **Target joints*** can lead to a need for surgery¹² and remain a **major cause of arthropathy and debilitating pain** for some people with hemophilia¹⁰
- A key component of comprehensive hemophilia care is the management of **musculoskeletal complications** and maintaining joint health¹
- Management of these joints requires both **timely diagnosis** and **early intervention**^{1,6}
- There is a need not only for **greater access to diagnostic tools**,¹³ but also for further developments in the **treatment of hemophilic arthropathy**⁵
- Currently, **no biomarkers** of joint deterioration have been validated or adopted for use in clinical practice¹³

*A joint that has three or more spontaneous bleeds within a continuous 6-month period.¹⁴¹ Srivastava A. Haemophilia 2024; 30:52–9. doi: 10.1111/hae.14958. Epub ahead of print; 2. Srivastava A et al. Haemophilia 2020;26:1–158; 3. Chhabra A et al. Blood Coagul Fibrinolysis 2020;31:186–92; 4. Waller J et al. 16th Annual Congress of the European Association for Haemophilia and Allied Disorders, February 7–10, 2023, Manchester UK. Poster PO199; 5. Oladapo AO et al. Orphanet J Rare Dis 2018;13:198; 6. Gualtierotti R et al. J Thromb Haemost 2021;19:2112–21; 7. Hermans C and Pierce GF. Haemophilia 2023;29:951–3; 8. Skinner MW et al. Haemophilia 2020;26:17–24; 9. World Federation of Hemophilia. WBDR 2022 DATA REPORT. Available at: <https://wfh.org/article/wbdr-2022-data-report-published/>. Accessed April 2024; 10. O'Hara S et al. Haemophilia 2021;27:113–9; 11. Warren BB. Blood Adv 2020;4:2451–9; 12. O'Hara J et al. Health Econ Rev 2018;8:1; 13. Cuesta-Barriuso R et al. J Blood Med 2022;13:589–601; 14. Blanchette VS et al. J Thromb Haemost 2014;12:1935–9.

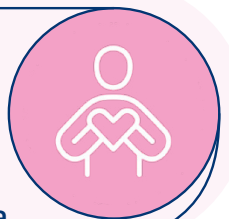
Enhancing quality of life and treatment experience



Quality of life

- Hemophilia can have a **significant effect** on the overall **quality of life** of people with hemophilia, with many people experiencing issues with **physical health**, **mental health** and **social challenges**¹
- The prevalence of **mental health** issues among people with hemophilia is **significantly higher** than the general population²

Recent real-world data suggest that up to **42% of people with hemophilia** in the USA reported experiencing **bruising**, **physical discomfort** and/or **pain** after treatment administration⁴



Treatment preparation and administration

- Some current therapies for hemophilia are associated with a **high burden of preparation and administration**, impacting adherence³
- Frequent **intravenous injections** can be **painful**, cause **scarring**, and are more difficult in patients with poor venous access³
- **Simplified dosing protocols**, **patient-friendly drug delivery systems**, and improvements in **treatment storage and portability** are all needed^{3,5}

Physical freedom

- People with hemophilia continue to report issues with **mobility and activity**,⁶ as well as **lower levels of physical activity** compared with the overall population⁷
- **Poor physical functioning** of people with hemophilia can **limit participation** in daily and recreational **activities**⁷
- Improved therapeutics have still not eradicated the **physical burden** experienced by some people with hemophilia⁸



School & work

- Children and adults with hemophilia can experience **absenteeism** in school or at work, impacting their **lifestyle, education, work, and productivity**^{9,10}
- Despite improvements in available therapies in hemophilia, an unmet burden on **school and work performance** persists^{9,10}



Among people with hemophilia, **80% report a negative impact of hemophilia on working life**¹¹

Unmet needs remain, and advancements in treatment and improved outcomes play an important role as we move towards health equity in hemophilia.^{1,7}