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]

Bleed protection

- Protection from **muscle and joint bleeds** is an important aspect of hemophilia care[1]
- 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 bleeds[4]
- People living with **hemophilia A and B with inhibitors** experience a **significantly greater burden of bleeding** than those without inhibitors[5]
- 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 bleeds[9]

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 surgery[12] and remain a **major cause of arthropathy and debilitating pain** for some people with hemophilia[10]
- A key component of comprehensive hemophilia care is the management of **musculoskeletal complications** and maintaining joint health[1]
- 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 practice[13]

*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.

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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 population[2]

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 administration[4]

Treatment preparation and administration

- Some current therapies for hemophilia are associated with a **high burden of preparation and administration**, impacting adherence[3]
- Frequent **intravenous injections** can be **painful**, cause **scarring**, and are more difficult in patients with poor venous access[3]
- **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 population[7]
- **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 hemophilia[8]

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]

1. Srivastava A. Haemophilia 2024; 30:52–9. doi: 10.1111/hae.14958. Epub ahead of print; 2. Al-Huniti A et al. Haemophilia 2020;26:431–42; 3. Okaygoun D et al. J Biomed Sci 2021;28:64; 4. Shah S et al. Hemostasis and Thrombosis Research Society Scientific Symposium, March 10–12, 2023, Orlanda, FL, USA. Poster P2.19; 5. Tischer B et al. Patient Prefer Adherence 2018;12:431–41; 6. Waller J et al. 16th Annual Congress of the European Association for Haemophilia and Allied Disorders, February 7–10, 2023, Manchester UK. Poster PO199; 7. Skinner MW et al. Haemophilia 2020;26:17–24; 8. Baumann K et al. Eur J Haematol 2017;98:25–34; 9. Mahlangu J et al. Haemophilia 2019;25:382–91; 10. Cutter S et al. Eur J Haematol. 2017;98:18–24; 11. Forsyth AL et al. Patient Prefer Adherence 2015;9:1549–60.

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