

HAEMOPHILIA IN EUROPE TODAY



HAEMOPHILIA IS A LIFELONG BLEEDING DISORDER, CAUSED BY A GENETIC MUTATION

- Haemophilia is a **rare genetic condition** caused by a mutation in the gene that provides instructions to make a protein called factor VIII or factor IX, which is essential for blood to clot normally.¹
- There are two main types of haemophilia:

Haemophilia A

occurs in 1 in 10,000 people

People with haemophilia A are missing or have low levels of **clotting factor VIII**.¹

Haemophilia B

occurs in 1 in 50,000 people

People with haemophilia B are missing or have low levels of **clotting factor IX**.¹

- Haemophilia can occur in a variety of severity levels, depending on the level of clotting factor in a patient's blood. This is classified as **mild, moderate** or **severe**.²

PEOPLE WITH SEVERE HAEMOPHILIA OFTEN LIVE WITH THE CONSTANT FEAR OF BLEEDING AND INJURY³

The symptoms of haemophilia include:



bruising easily¹



bleeding into muscles and joints¹



prolonged bleeding after getting a cut – making fear of injury a constant feature of life for people living with severe haemophilia¹

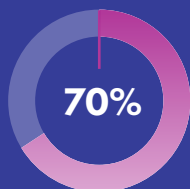


spontaneous bleeding, where bleeding occurs suddenly within the body for no clear reason¹

For patients who continue to experience spontaneous bleeds, the condition can be life-threatening.³

SEVERE HAEMOPHILIA HAS A SIGNIFICANT IMPACT ON PATIENTS' QUALITY OF LIFE

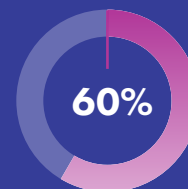
Despite the current treatment options available to patients, many people with haemophilia continue to experience bleeds, which can cause progressive damage to their joints.^{5,6} This can result in people being unable to take part in day-to-day activities, suffering from depression or anxiety, and living a life in which pain is a constant feature.⁷



70% of **bleeding episodes** for people with severe haemophilia **occur in joints**, leading to damage over time.⁸



2/3 of people living with severe haemophilia **experience pain daily**.⁸

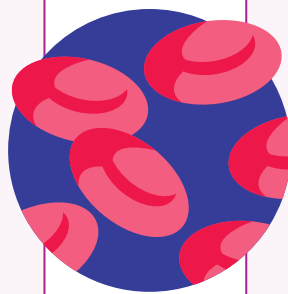


60% of people living with haemophilia report that **painful symptoms are a direct cause of "low spirits"**.⁹

PEOPLE LIVING WITH HAEMOPHILIA FACE A TREATMENT BURDEN

Severe haemophilia comes with a treatment burden, which can have a psychological impact on both patients and caregivers.^{9,10}

People living with haemophilia need to **replace the clotting factor they are missing**. In the past, clotting factor was derived from pooled human plasma from blood donors. Today, many people living with haemophilia are also treated with recombinant, or synthetic, clotting factor replacement therapy, although this can be unavailable in countries with limited health resources.¹¹



People living with severe haemophilia typically **require routine intravenous injections 2-3 times per week** to maintain target levels of clotting factors, and many struggle to commit to this time-intensive routine.^{10,12} Even on newer non-factor replacement therapies, sub-cutaneous injections can still be **required once a week, twice a month** or monthly depending on the patient.¹⁰

REFERENCES

- ¹ World Federation of Haemophilia. Introduction to Haemophilia. Available at: https://elearning.wfh.org/elearning-centres/introduction-to-hemophilia/#what_is_hemophilia Last Accessed September 2021
- ² Benson G. et al. Diagnosis and care of patients with mild haemophilia: practical recommendations for clinical management. *Blood Transfus.* 2018;16:535-44
- ³ O'Hara, J. et al. 'The cost of severe haemophilia in Europe: the CHESSE study'. *Orphanet Journal of Rare Diseases*, 12:106. 2017
- ⁴ Franchini, M. et al. Past, present and future of hemophilia: a narrative review. *Orphanet J. Rare Dis* 2012;7:24.
- ⁵ duTreil, S. Physical and psychosocial challenges in adult hemophilia patients with inhibitors. *J. Blood Med* 2014;5:115-122.
- ⁶ Manco-Johnson M et al. Prophylaxis versus Episodic Treatment to Prevent Joint Disease in Boys with Severe Hemophilia. *N Engl J Med* 2007;357:535-544.
- ⁷ O'Hara, J et al. Associated impact of activity impairment on clinical and patient-centric outcomes in severe hemophilia. *Haemophilia* 2018;24:1.
- ⁸ Auerswald G. et al. Pain and pain management in haemophilia. *Blood Coagul Fibrinolysis.* 2016 Dec; 27(8): 845-854.
- ⁹ Batt K. et al. Measurement of Pain and Health-Related Quality of Life Measures in Adult Males with Hemophilia: Initial Observations of Disease Impact (S724). *J. Pain Symptom Manag.* 2016;51:2
- ¹⁰ Callaghan MU, Negrier C, Paz-Priel I, et al. Long-term outcomes with emicizumab prophylaxis for hemophilia A with or without FVIII inhibitors from the HAVEN 1-4 studies. *Blood.* 2021;137(16):2231-2242. doi:10.1182/blood.202009217
- ¹¹ Noone D et al. Evolution of Haemophilia Care in Europe: 10 years of the principles of care. *Orphanet J. Rare Dis* 2020; 15:184. <https://doi.org/10.1186/s13023-020-01456-y>
- ¹² Srivastava A et al. Guidelines for the management of Hemophilia. *Haemophilia* 2013;19(1):e1-47.