TREATMENTS FOR HAEMOPHILIA

ACE agency for care effectiveness

WHAT IS HAEMOPHILIA?

Haemophilia is a **life-long** condition where **blood does not clot properly**. It is often **passed down** from a parent to a child and is caused by a lack of proteins (**clotting factors**) in the blood that control bleeding.[1]

There are two main types of haemophilia:

HAEMOPHILIA A

The **most common** form caused by having low levels of **clotting factor eight (VIII)**

HAEMOPHILIA B

A **less common** form caused by having low levels of **clotting factor nine (IX)**

Haemophilia mainly affects **males**[2]

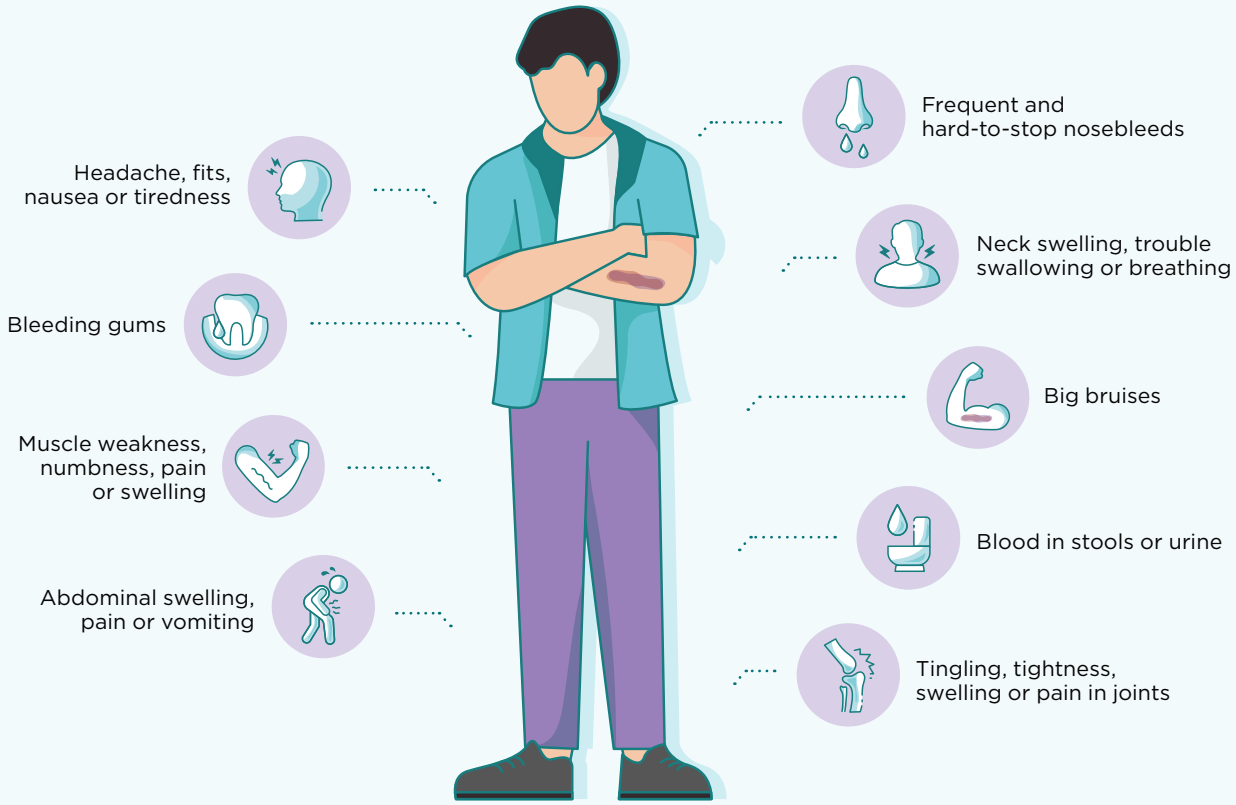
In Singapore:

About **250** people are living with **haemophilia A**

About **50** people are living with **haemophilia B**

People with haemophilia do not bleed faster but can bleed **longer** than other people. Bleeding can occur **inside or outside the body** due to injury, dental treatment, surgery or without any obvious cause.

Symptoms can range from being mild to serious depending on how much clotting factor is missing and **where** the bleeding occurs:



HOW IS HAEMOPHILIA TREATED?

Haemophilia cannot be cured, but effective treatments are available to treat and prevent bleeding and allow people to live **normal** lives. Treatments can be given **regularly** or **when needed**.

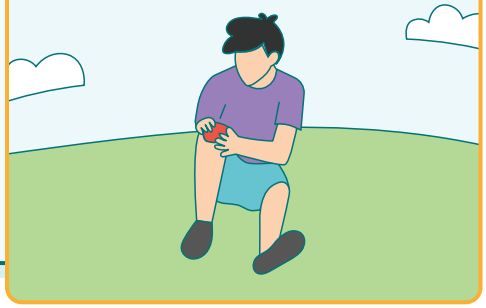
The **choice** of treatment depends on **different reasons** such as how severe the bleeding is; whether you need to have treatment regularly or when needed; if you have developed antibodies (inhibitors) that stop certain clotting factors from working; as well as your **personal needs** and **preferences**.

Regularly (prophylaxis)

To **prevent** bleeding before it starts

When needed (on-demand)

To **treat** bleeding when it happens, or before surgery or dental treatment[3]



TREATMENTS FOR HAEMOPHILIA

Clotting Factor Replacement Therapies

Treatments that **replace the clotting factor** that is low or missing in the blood to help blood clot properly and prevent or reduce bleeding.

Plasma-derived products made from donated blood

- Plasma-derived factor VIII
- Plasma-derived factor IX

Recombinant factor concentrates made in a laboratory and not from blood

	Standard half-life (Lasts for 8-12 hours in the body)	Extended half-life (Lasts longer in the body)
Factor VIII	• Advate • Nuwiq • Novoeight • Xyntha	• Adynovate • Afstyla
Factor IX	• BeneFIX	• Idelvion

Given slowly into a vein (intravenously)

Other Therapies

Treatments that help blood to clot **without replacing the clotting factor**. These are usually given to people who develop inhibitors to clotting factors.

- Factor VIII Inhibitor Bypassing Activity (FEIBA)
- Recombinant factor VIIa (NovoSeven)

Given slowly into a vein (intravenously)

- Emicizumab

Given as an injection under the skin (subcutaneously)

Key: Factor VIII, Factor eight; Factor IX, Factor nine; Factor VIIa, Activated Factor seven.

WHAT DOES THE EVIDENCE FROM CLINICAL STUDIES SAY?

Clotting Factor Replacement Therapies

Plasma-derived products and **recombinant factor concentrates** are effective treatment options for **haemophilia A and B** without inhibitors.

Other Therapies

FEIBA and **NovoSeven** are effective treatment options for **haemophilia A and B** with inhibitors. **Emicizumab** is also an effective option for treating **haemophilia A** with or without inhibitors.

SUBSIDISED TREATMENTS

ACE reviewed all available clinical evidence and received clinical advice from doctors about each treatment. ACE also negotiated prices with the companies.

SUBSIDISED

These treatments are **effective** and provide the best value for money (most cost-effective) for haemophilia at the prices proposed by the companies.[4,5]

- Plasma-derived factor VIII
- Recombinant factor VIII (Adynovate, Advate and Xyntha)
- Recombinant factor IX (BeneFIX)
- Factor Eight Inhibitor Bypassing Activity (FEIBA)

Treatment costs are subsidised by **50% to 75%** for eligible patients

NOT SUBSIDISED

These treatments were not subsidised because they are not commonly used to treat haemophilia in Singapore or their benefits do not justify their costs at the prices offered by the companies.

- Emicizumab
- Recombinant factor VIII (Afstyla, Novoeight and Nuwiq)
- Recombinant factor VIIa (NovoSeven)
- Plasma-derived factor IX
- Recombinant factor IX (Idelvion)

KEY MESSAGES

- **Plasma-derived factor VIII** and **recombinant factor VIII** (Adynovate, Advate and Xyntha) are subsidised for treating haemophilia A
- **Recombinant factor IX** (BeneFIX) is subsidised for treating haemophilia B
- **Factor Eight Inhibitor Bypassing Activity** (FEIBA) is subsidised for treating haemophilia A and B with inhibitors

The choice of treatment depends on different reasons. Talk to your **doctor** to discuss which treatment is suitable for you. You can also speak to a **medical social worker** if you need further financial assistance, or you can reach out to **local patient support groups**[6] if you want to meet people with haemophilia and share your experiences.

Sources

1. https://www.singhealth.com.sg/patient-care/conditions-treatments/haemophilia-childhood-illnesses
2. Report on the Annual Global Survey 2021, World Federation of Haemophilia, 2022.
3. https://www.nuh.com.sg/Health-Information/Diseases-Conditions/Pages/Haemophilia-(Children).aspx
4. ACE Technology Guidance, Emicizumab prophylaxis for patients with haemophilia A, 1 July 2022.
5. ACE Technology Guidance, Recombinant blood products for prophylaxis and management of haemophilia A and B, 7 December 2022.
6. Haemophilia Society of Singapore (HSS); Promisedland Community Services (PCS)

Published on 16 March 2023. To find out more about ACE, scan the QR code or visit **www.ace-hta.gov.sg**. You can also follow us on social media at: