

What is haemophilia A?

Haemophilia is an inherited, **serious bleeding disorder** where a person's **blood does not clot properly**, leading to uncontrolled bleeding which can occur spontaneously or after minor trauma.

It can **dramatically reduce the quality of life** of people affected, as well as their family, friends and caregivers¹.

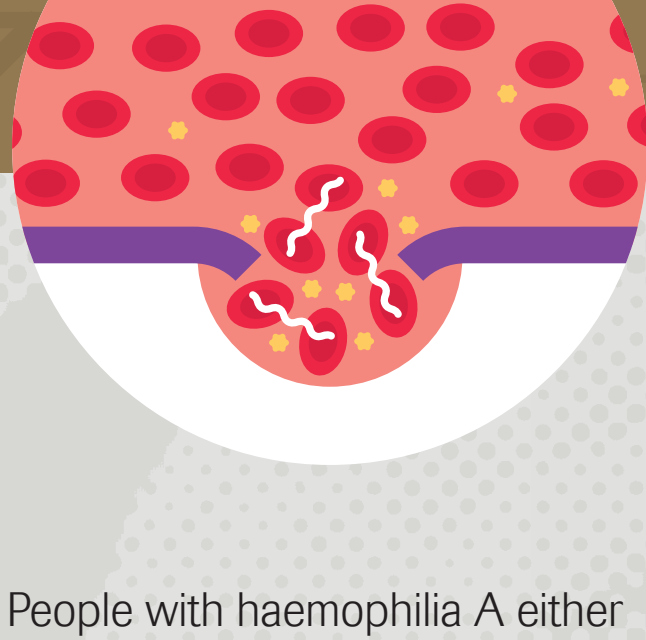
Haemophilia A is the most common form – affecting

~320,000 people worldwide^{2,3}



50-60% of whom have severe haemophilia⁴.

What happens in the blood of a person with haemophilia A?



In a healthy person, proteins called **clotting factors** work together to form a blood clot and help stop bleeding.

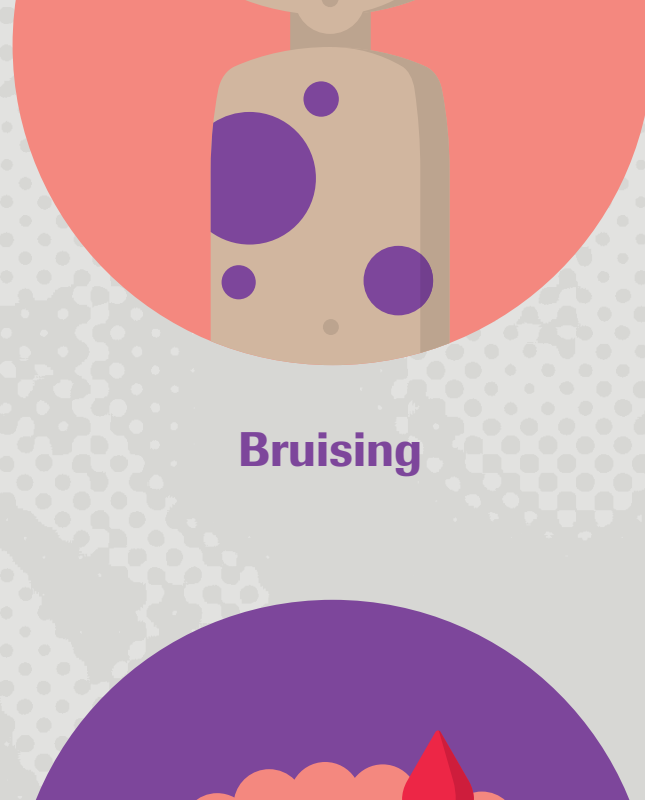
People with haemophilia A either **lack or do not have enough** of a clotting factor called

factor VIII

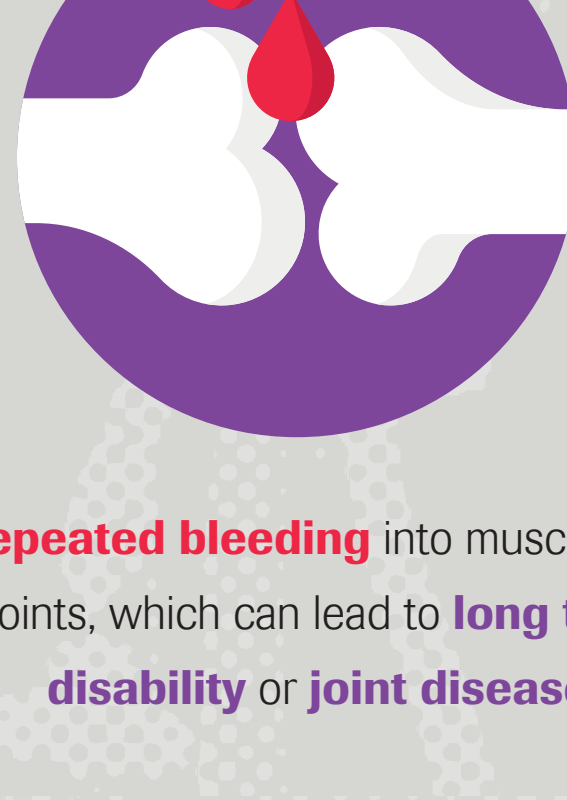
which leads to their blood not being able to clot properly.



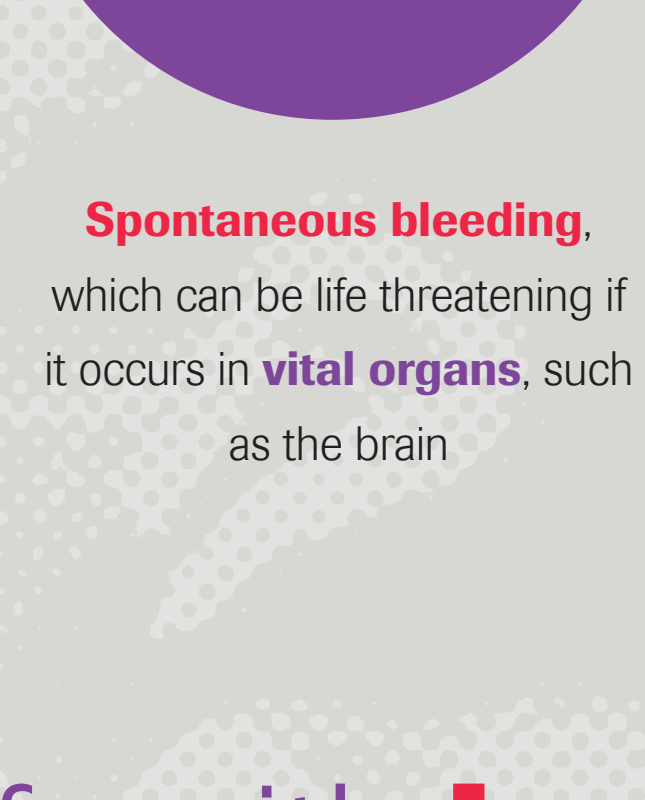
Without treatment, people with haemophilia can suffer:



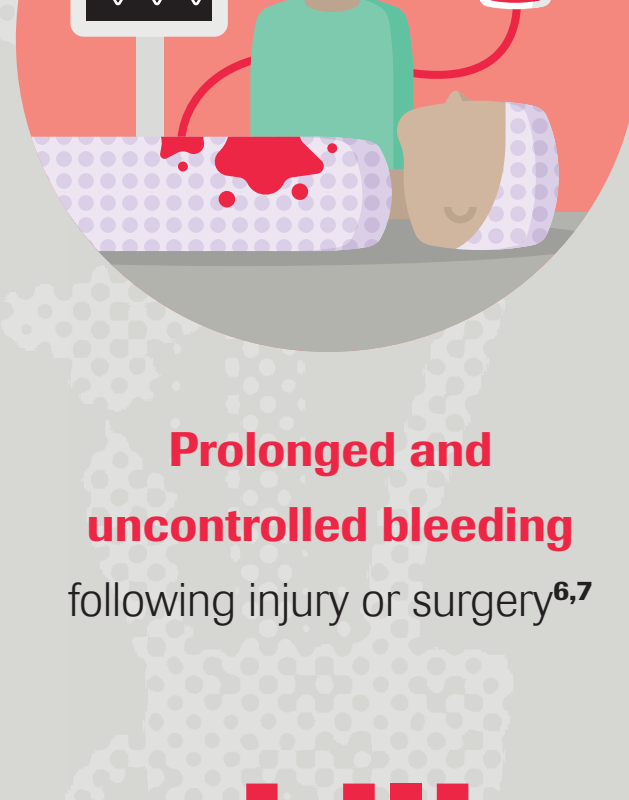
Bruising



Repeated bleeding into muscles and joints, which can lead to **long term disability** or **joint disease**⁵

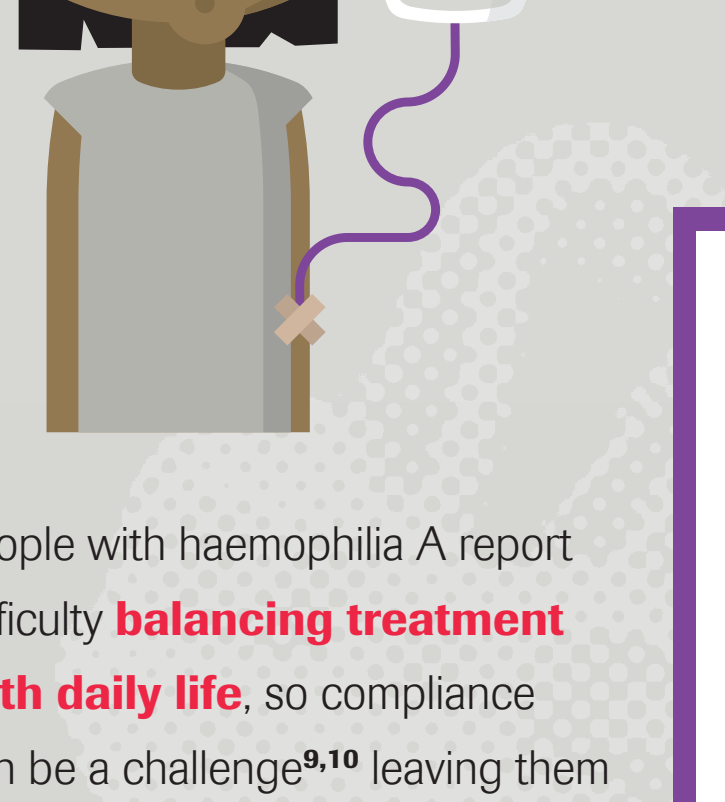


Spontaneous bleeding, which can be life threatening if it occurs in **vital organs**, such as the brain



Prolonged and uncontrolled bleeding following injury or surgery^{6,7}

Life with haemophilia – the burden of treatment:



People with haemophilia A report difficulty **balancing treatment with daily life**, so compliance can be a challenge^{9,10} leaving them **vulnerable to potentially dangerous bleeds**.

Life for people with haemophilia and their caregivers is often **centred on treatment infusions**, taking up a large amount of time and having a **significant impact on their lives**⁸.



Factor VIII replacement

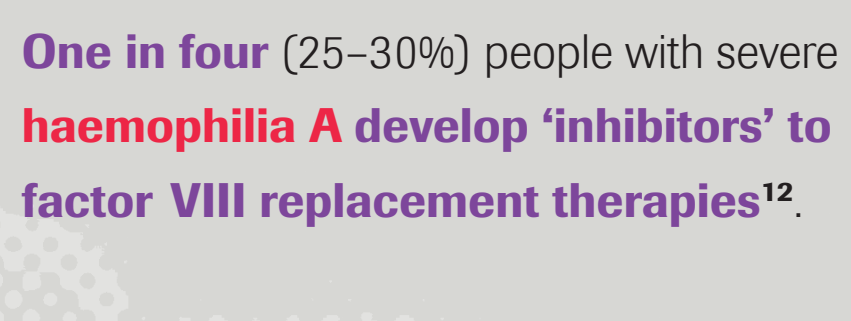


The mainstay of current treatment for haemophilia A **is factor VIII replacement therapy**, which is taken **on-demand** (as needed to treat bleeds), or on an **ongoing basis** (to prevent bleeds).

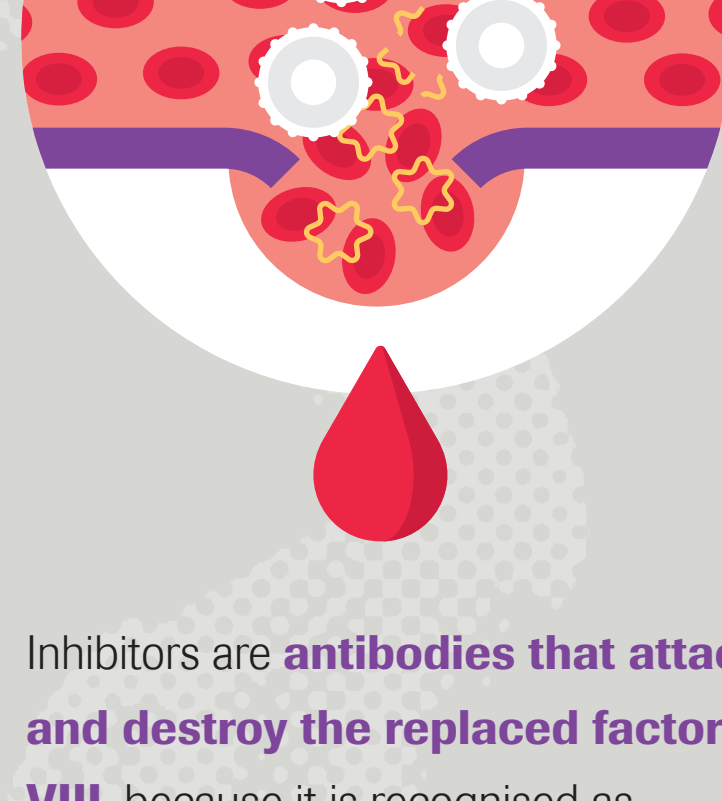
It is **short-acting** and so needs to be **administered frequently** (at least twice a week)² **by the patient or a caregiver** and for some, especially children, finding a vein for medicine infusion can be difficult¹¹.



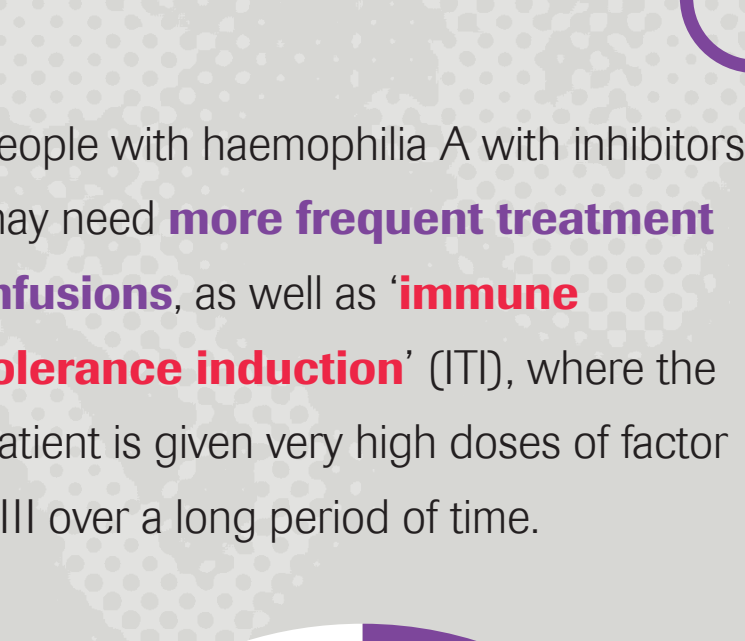
Treating inhibitors: ITI and bypassing agents



One in four (25–30%) people with severe **haemophilia A** develop **'inhibitors'** to **factor VIII replacement therapies**¹².



Inhibitors are **antibodies that attack and destroy the replaced factor VIII**, because it is recognised as foreign¹³. As a **serious complication** of treatment¹⁴, many people with haemophilia A **live in fear** of developing inhibitors.



People with haemophilia A with inhibitors may need **more frequent treatment infusions**, as well as **'immune tolerance induction'** (ITI), where the patient is given very high doses of factor VIII over a long period of time.



However, ITI can take many years, is very costly and is **ineffective in ~30%** of people^{15,16}.

'Bypassing agents' are another treatment for people with inhibitors, often used after ITI fails. However, these are **short-acting, needing to be taken often and give variable bleeding control**¹⁷.

Further effective and safe treatment options for people with **haemophilia A** are needed to enable them to better manage their condition and live their lives with **less burden from treatment**

References

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