

Plain English Summary

Emicizumab prophylaxis for patients with haemophilia A

What does the guidance say?

Emicizumab is not recommended for government subsidy as prophylaxis for patients with haemophilia A to prevent or reduce bleeding.

Why was emicizumab not recommended for subsidy?

ACE evaluates how well a treatment works in relation to how much it costs compared to other treatments. Emicizumab was not recommended for government subsidy because its benefits for patients with haemophilia A do not justify its cost. If you need emicizumab for haemophilia A, you can speak to a medical social worker to find out if there is other financial assistance available to help with the cost of treatment.

What is haemophilia A?

Haemophilia A (also known as classic haemophilia) is a rare, life-long condition that affects the blood's ability to clot. It is usually inherited (passed from parents to children) and is most common in males.

Normally, when you cut yourself, clotting proteins help make blood sticky and form a clot to stop any bleeding. However, people with haemophilia A have a lack of clotting factor eight (VIII) proteins so they bleed longer than usual. They can have internal bleeding into joints and muscles, or external bleeding due to injury, dental procedures or surgery. How often someone with haemophilia A bleeds and how severe the bleeds are, depend on how much factor VIII they produce naturally.

Most treatments for haemophilia A aim to replace the missing factor VIII and prevent bleeds or reduce how often they occur. Patients may be treated during a bleeding episode or surgery (on-demand) or have regular treatment (prophylaxis) to prevent bleeding.

What is emicizumab?

Emicizumab belongs to a group of medicines called recombinant monoclonal antibodies. It mimics and restores the function of the missing factor VIII in patients with haemophilia A to prevent bleeding or help reduce how often bleeding occurs. It is given as an injection under the skin.

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Is this the right treatment for me?

There are different types of treatments available for haemophilia A. Your doctor may recommend you have emicizumab if other drugs cannot adequately control your condition. Your doctor should give you clear information, listen to your views and concerns, and talk to you about your treatment options.

Some of the questions you may want to ask your doctor when making decisions about your care are:

- How will the treatment affect my day-to-day activities?
- How well does it work compared to other treatments?
- What are the side effects and risks of treatment, and how likely are they?
- How long will I need to be on the treatment for?
- What happens if the treatment stops working?
- What happens if I do not want to have treatment?

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The Agency for Care Effectiveness (ACE) was established by the Ministry of Health (Singapore) to drive better decision-making in healthcare through health technology assessment (HTA), clinical guidance and education. It publishes guidances on diagnosing, treating, and preventing different medical conditions based on the latest research information available worldwide.

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