The Scottish Medicines Consortium (SMC) has assessed caplacizumab for the treatment of adult patients who have an episode of acquired thrombotic thrombocytopenia purpura (aTTP). It is used together with plasma exchange and immunosuppression. This document summarises the SMC decision and what it means for patients.

What has SMC said?

After careful consideration, SMC has accepted caplacizumab for the treatment of aTTP as described above.

This advice takes into account a confidential discount offered by the pharmaceutical company that improves the cost-effectiveness of caplacizumab.

What is caplacizumab used for?

Caplacizumab is used to treat an episode of aTTP, which is a rare blood clotting disorder. An episode of aTTP leads to small blood clots forming in blood vessels, low platelet counts and the breakdown of red blood cells. Caplacizumab is used to treat episodes of aTTP together with plasma exchange (where certain antibodies are removed from the blood) and treatments to suppress the immune system.

How does caplacizumab work?

Caplacizumab acts to block the interaction between a substance called von Willebrand factor (vWF) and platelets. In patients with aTTP, vWF is increased which causes platelets to stick together and form clots. By blocking the interaction between vWF and platelets, caplacizumab helps stop platelets sticking together, helping prevent the formation of blood clots that can cause tissue damage and organ failure in aTTP.
How does SMC make its decision?
SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland.

To do this SMC consider the following:

- Evidence from the company about how well the medicine works compared with current treatments available in Scotland, in relation to how much they will cost to buy and administer.
- The potential impact of the medicine on patients and carers.
- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.

When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not just those who may be treated with the medicine under consideration.

You can find more detailed information about the SMC assessment of caplacizumab by looking at the SMC Detailed Advice Document (SMC2266).

More information

The organisations below can provide more information and support for people with aTTP and their families. SMC is not responsible for the content of any information provided by external organisations.

Thrombosis UK

https://www.thrombosisuk.org  0300 772 9603

TTPNetwork

https://www.ttpnetwork.org.uk

You can find out more about caplacizumab (Cablivi®) in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.

http://www.ema.europa.eu