Plain English Summary

Complement inhibitors for treating paroxysmal nocturnal haemoglobinuria and atypical haemolytic uraemic syndrome

What does the guidance say?

Eculizumab is recommended for listing on the Medication Assistance Fund (MAF) for government subsidy for treating adults and children with paroxysmal nocturnal haemoglobinuria (PNH) or atypical haemolytic uraemic syndrome (aHUS) who meet certain clinical criteria.

Iptacopan and ravulizumab are not recommended for subsidy for treating PNH. Ravulizumab is also not recommended for subsidy for treating aHUS.

What is paroxysmal nocturnal haemoglobinuria?

Paroxysmal nocturnal haemoglobinuria (PNH) is a rare blood disorder that develops over time due to a change (mutation) in a gene in the bone marrow. This gene mutation happens on its own (acquired) and is not inherited from parents.

In PNH, the bone marrow produces abnormal red blood cells that are missing a protective layer on their surface. Without protection, part of the body's immune system called the complement system can mistakenly attack and destroy these red blood cells.

Haemoglobin is a protein in red blood cells that helps carry oxygen around the body. In PNH, red blood cells break down earlier than they should, releasing haemoglobin into the blood and urine. This can cause episodes of dark-coloured urine, which often occur at night or in the early morning. Other symptoms of PNH may include fatigue due to low red blood cell levels (anaemia), shortness of breath, difficulty swallowing, blood clots, stomach pain and kidney damage. PNH can occur at any age, but it is most commonly diagnosed in young adults.

What is atypical haemolytic uraemic syndrome?

Atypical haemolytic uraemic syndrome (aHUS) is a rare blood disorder that can affect both adults and children. It occurs when the complement system becomes overactive and damages blood vessels. This causes small blood clots to form, which can block blood flow to the kidneys and other organs, and can damage other blood cells as they pass through. In many people, aHUS develops due to changes in genes that help control the complement system. These gene changes can develop on their own (acquired) or be inherited from parents, and in some cases, aHUS can be triggered by infections, surgery or certain medicines.

People with aHUS typically develop anaemia, low platelet count and kidney failure. Other symptoms include high blood pressure, swelling, confusion and stroke.

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What is eculizumab?

Eculizumab belongs to a group of medicines called complement inhibitors that block the overactive complement system. This prevents the damage of red blood cells and the formation of blood clots. It is given as a slow drip into a vein (intravenously). Your doctor will tell you how much you need and how long you need to have it for.

Who can have eculizumab?

Eculizumab is used to treat people with PNH or aHUS who meet certain clinical criteria. Your doctor will advise if eculizumab is a suitable treatment for you.

Why was eculizumab recommended for subsidy?

ACE evaluates how well a treatment works in relation to how much it costs compared to other treatments. Eculizumab was recommended for government subsidy as it was considered to be an acceptable use of healthcare resources for treating adults and children with PNH or aHUS.

Iptacopan and ravulizumab were not recommended for subsidy as their benefits do not justify their costs compared with eculizumab. You can speak to a medical social worker to find out if there is financial assistance available to help with the cost of these treatments if you need them.

What does listing on the MAF mean for me?

The MAF helps people pay for treatments that are clinically effective and cost effective. If your doctor prescribes eculizumab for you, and you meet the MAF criteria, your treatment cost will be subsidised by 40% to 75%.

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