National Centre for Pharmacoeconomics
NCPE Ireland

NCPE Plain English Summary

Drug name: Ravulizumab *(pronounced: RAV-ul-IZ-u-mab)* for the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH), in patients with haemolysis with clinical symptom(s) indicative of high disease activity; and in patients who are clinically stable after treatment with eculizumab for at least the past six months.

Brand name: Ultomiris®

What is the NCPE?

The National Centre for Pharmacoeconomics (NCPE) is a team of experts who look at the health benefits and costs of medicines. The HSE asks us to advise on whether or not a new medicine is good value for money. We give unbiased advice to help the HSE provide the most effective, safe and cost-effective (value for money) treatments for patients.

How do we make our recommendations?

Our main focus is on the health benefits and cost effectiveness of a medicine. We look at the wider costs and health benefits associated with a new medicine, for example:

- Does the new medicine work better than other treatments available in Ireland?
- Is the new medicine easier to give or easier to take compared with other treatments available in Ireland?
- Does the new medicine reduce the need for patients to be hospitalised?
- Does the new medicine improve the quality of a patient's life over other treatments available in Ireland?
- Will the new medicine save resources elsewhere within the health system?

We review the information from clinical trials along with the cost and value for money data presented by the pharmaceutical company. We ask doctors and other healthcare professionals for advice about any health benefits of the new medicine compared with current treatments. We also ask patient organisations to send us their views on how the new drug may improve patients' day-to-day experience of living with a disease.

What is ravulizumab used for?

Ravulizumab is a medicine used to treat paroxysmal nocturnal haemoglobinuria (PNH), which is a rare life-threatening disease of the blood causing destruction of red blood cells, blood clots, and impaired bone marrow function. It is used in adults with active PNH disease and in those who are stable after at least six months of treatment with eculizumab, which is the current treatment for PNH disease.

What recommendation has the NCPE made to the HSE?

We have recommended that the HSE should consider funding ravulizumab, provided it does not cost more than eculizumab products currently available or anticipated to become available in the near future. The HSE will consider our recommendation and make the final decision about reimbursement (funding). When making the funding decision, the HSE will also consider the additional criteria outlined in the Health (Pricing and Supply of Medical Goods) Act 2013.

Why did we make this recommendation?

After reviewing the data presented by the pharmaceutical company, we recommend that the HSE consider providing ravulizumab if the HSE can agree a suitable price reduction with the pharmaceutical company. We believe the medicine works similarly to the current treatment for this condition. However, the price of the medicine is too high compared with anticipated treatments on the horizon. The HSE considers a number of factors along with our recommendation when deciding whether to provide this medicine. These factors are listed in the Health (Pricing and Supply of Medical Goods) Act 2013

Next steps

When the HSE receives our recommendation, it will look at all the relevant data about ravulizumab. The HSE makes the final decision on reimbursement.

Where can I get more information?

You can get more information about ravulizumab from the following online options:

- the NCPE Technical Summary Document
- ravulizumab European Public Assessment Report (EPAR) – Summary for the public
- searching for ravulizumab on our website (www.ncpe.ie) or the European Medicines Agency (EMA) website (www.ema.europa.eu).

Please refer to the NCPE website for updated information on the reimbursement status of this medicine.

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