NOTIFICATION

PHARMAC
Pharmaceutical Management Agency

12 December 2013

PHARMAC decision on eculizumab (Soliris) funding

PHARMAC has declined the funding application for eculizumab for paroxysmal nocturnal haemoglobinuria (PNH), a rare blood disorder. The main reason for this decision is that the price being sought by Alexion Pharmaceuticals (the supplier) is too high for PHARMAC to justify funding in light of other available funding options.

This was the subject of a consultation letter dated 21 May 2013 which can be found at: http://www.pharmac.health.nz/ckeditor_assets/attachments/362/consultation-2013-05-21-eculizumab.pdf

PHARMAC recognises the clinical benefit of eculizumab and the high health needs of patients with PNH. While the funding application has been declined, PHARMAC is open to considering a future funding application if a new commercial proposal is received from Alexion.

Reasons for decision

High price

The decision to decline funding is primarily because the price of eculizumab is extreme, which means that its cost effectiveness is low relative to other active funding applications for other medicines we are considering. We note that the price offered by Alexion is higher than it charges in other countries (even taking into account the discounts it offered), and out of line with other comparable innovative new medicines supplied by other companies.

Cost effectiveness

Even if we assumed that eculizumab was 100% effective and guaranteed patients were restored to full health with normal life expectancy, at the price offered, eculizumab would still be about 20 times less cost effective than the average medicine funded by PHARMAC over the past two years. However, a 100% effectiveness assumption is not supported by clinical trials and the other evidence available. Alexion has been unwilling to offer a price that would bring eculizumab within an acceptable cost effectiveness range.

Comparisons with other treatments

PHARMAC funds a number of treatments for rare diseases which are life-threatening. In those cases, the suppliers have offered prices that more closely reflect the benefits of the treatment.

For example, eltrombopag, supplied by GlaxoSmithKline and recently funded by PHARMAC, is an innovative medicine which treats another rare blood disorder and costs $36,000 (excluding confidential rebates) per patient per year; a price which makes it one of the more expensive per-patient medicines funded by PHARMAC. Eculizumab is priced at about $670,000 per patient per year, making it about 20 times the price on a per-patient basis.

Level 9, 40 Mercer Street, PO Box 10-254, Wellington 6143, New Zealand
Phone: 64 4 460 4990 - Fax: 64 4 460 4995 - www.pharmac.govt.nz

Deferiprone, supplied by Healthcare Logistics, is another product funded by PHARMAC for a rare blood disorder. About 33 patients with transfusional iron overload, due to congenital inherited anaemias (mainly thalassaemia), use this medicine at an annual cost of only $8,000 (excluding confidential rebates) per patient.

When assessing the eculizumab funding application PHARMAC also considered the inequity that the impact of funding it would have on a number of other active funding applications for medicines that, following consideration of all of PHARMAC's decision criteria, are considered a higher priority for funding than eculizumab. These medicines are, in effect, ahead of eculizumab in the 'queue' for funding at this time.

Impact on patients

We are very aware of the impact of this decision on patients with PNH in New Zealand. Eculizumab has benefits for patients and, although the long-term benefit has not been established, PHARMAC would like to be able to fund it. However, the price being asked by Alexion puts funding far out of our reach, and would impact the availability of medicines for other patients.

PHARMAC operates within a fixed budget, and needs to make funding choices on how to use the budget for all New Zealanders. Eculizumab could benefit up to 20 people, at a cost of approximately $10 million per year. If funding was committed to the drug, it would mean potentially tens of thousands of New Zealanders missing out on new medicines which offer more health gain overall.

In the 2012/13 financial year, PHARMAC spent $17.5 million on new investments (new treatments and widening access to existing treatments) which benefited an estimated 52,400 patients.

Future consideration

This decision to decline funding for eculizumab does not mean the door is closed to eculizumab funding. PHARMAC is prepared to reconsider eculizumab funding if new information, in particular more reasonable pricing, becomes available. New pricing would need to more closely reflect the benefits and be in line with the cost effectiveness offered by other funding options.

Patients with PNH will continue to receive currently funded treatments and services including anticoagulation, blood transfusions and their regular clinical monitoring and review.

Background

PHARMAC received an application to fund eculizumab (Soliris) for the treatment of PNH from Alexion Pharmaceuticals in November 2011. PNH is a rare blood disorder that is characterised by the destruction of red blood cells, an increased risk of blood clots, impaired bone marrow function and a risk of developing leukaemia. Eculizumab is a monoclonal antibody that aims to stop this red blood cell destruction.

PHARMAC consulted on a proposal to decline the funding application for eculizumab in PNH in May 2013.

Feedback received

We appreciate all of the feedback that we received and acknowledge the time people took to respond. All 263 consultation responses received were considered in their entirety in making a decision on the proposed changes.

The following key themes were raised in the consultation responses:

- Impact on patients with PNH and their carers
- Quality of information provided by the consultation document
- Equity of access to treatment
- Special decision criteria required
- International funding of eculizumab
- Prioritisation of spending in New Zealand
- Suggested approaches to achieve funding for eculizumab
- Opportunity cost and fiscal risk
- Uncertainty of clinical benefit
- Role of the pharmaceutical industry

A summary of the consultation responses received can be found on our website at: http://www.pharmac.health.nz/ckeditor_assets/attachments/100/eculizumab-2013-08-analysis-of-feedback.pdf

We intend to release a list of submitters and copies of the submissions once we have processed these for public release including, where appropriate, consulting with submitters.

More information about the issues raised can also be found in the Board decision paper: http://www.pharmac.health.nz/ckeditor_assets/attachments/100/eculizumab-2013-11-board-decision-paper.pdf

More information

If you have any questions about this decision, you can email us at enquiry@pharmac.govt.nz or call our toll free number (9 am to 5 pm, Monday to Friday) on 0800 66 00 50.