aHUS ACTION

aHUS Action response to NICE ECD: Eculizumab for the treatment of atypical haemolytic uraemic syndrome

Has all of the relevant evidence been taken into account?

Yes. The report provides a comprehensive review of all the relevant literature.

Are the summaries of clinical effectiveness and value for money reasonable interpretations of the evidence?

The summary of clinical effectiveness is we believe a very reasonable interpretation of the evidence. Because the manufacturer has not provided an adequate explanation for the cost of eculizumab it is difficult to comment on the value for money.

Are the provisional recommendations sound and a suitable basis for guidance to NHS England?

It is not possible to comment on this because as stated in the document (section 1.2): "The Committee is therefore currently unable to prepare a recommendation on the use eculizumab for the treatment of aHUS and has asked for further information from the company to enable it to do so (section 5.8). It has also asked for advice from NHS England on what considerations relating to the management of its specialised commissioning budget it considers should be taken into account in formulating a recommendation."

We would like to make the following additional points.

1. We agree completely that the manufacturer must provide adequate information to justify the cost of eculizumab. Because both the current indications for eculizumab (PNH and aHUS) are ultra-rare diseases, it is obvious that the cost of eculizumab is going to be high, but does it have to be as high as at present? We agree with NICE that Alexion must provide the necessary information to justify the current cost.

2. We also agree with the request for advice from NHS England on "what considerations relating to the management of its specialised commissioning budget it considers should be taken into account in formulating a recommendation". We believe that an indication from NHS England as how much it can afford to spend on the use of eculizumab in PNH and aHUS is necessary for NICE to formulate an appropriate recommendation.

3. We believe that the introduction of a patient access scheme for both PNH and aHUS could address the affordability issue. We would encourage Alexion and NICE/DoH/NHS England to consider this.

4. We believe that there are also other ways in which the affordability issue can be addressed. We, as clinicians responsible for the care of aHUS patients, are of the opinion that eculizumab may not need to be given long-term for all patients. We believe that there are groups of patients in whom eculizumab could be safely withdrawn with a risk of recurrent disease that is appropriately low. We wish to study this in rigorous prospective trials but would not want to undertake this without the ability to restart the drug if necessary. The current NHS England Clinical Commissioning Policy for the use of eculizumab in aHUS does not afford us the security to undertake such studies. Once we have identified which patients need long-term treatment with eculizumab, we are also of the opinion that it may be possible in individual patients to either reduce the dosage and/or decrease the frequency of treatment whilst maintaining adequate complement blockade. As clinicians we are conscious of the fiscal responsibility that we have. We believe that there are ways in which we can reduce the cost to the NHS of using eculizumab in aHUS but this can only be done within an environement where long-term access to the drug, if necessary, is available.

5. An application for the use of eculizumab in aHUS was first submitted to AGNSS in December 2010. Since then, aHUS patients, their families and carers through no fault of their own have been caught in a protracted period of uncertainty. We believe that Alexion, NICE, NHS England and the Department of Health have the collective ability to end this uncertainty. We encourage them to do so.