Kidney Research UK Submission:
ECD: Atypical haemolytic uraemic syndrome (aHUS) - eculizumab [703]

Kidney research UK notes the recent ECD on Atypical haemolytic uraemic syndrome (aHUS) - Eculizumab [703]. This document duplicates the previous evaluation by AGNSS confirming Eculizumab's clinical effectiveness in aHUS.

Has all the evidence been taken into account?

The evidence of clinical effectiveness used in the appraisal seems appropriate and complete.

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

Kidney Research UK is not in a position to evaluate whether eculizumab is value for money. We note the lack of disclosure by Alexion pharmaceuticals as to the cost of Eculizumab relative to other monoclonal antibodies. Clinical trials of Eculizumab in other complement mediated conditions are underway and will likely provide an additional market to further dilute the development costs. We note the lack of guidance by NHS England as to what would constitute a reasonable cost. Without the necessary reference frame and complete disclosure, Kidney Research UK is unable to comment on value for money.

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

Kidney Research UK notes that both the AGNSS review and the subsequent NICE review concur as to the clinical effectiveness of Eculizumab for aHUS. It is a matter of concern to Kidney Research UK that, despite the initial positive AGNSS review in 2010, no long term agreement as to the use of Eculizumab in aHUS has been reached.

Research funded by Kidney Research UK in Newcastle has been central to understanding aHUS and ultimately to finding an effective treatment of the disease. We believe that funding research into rare kidney diseases improves patients' lives. It is with a sense of pride that Kidney Research UK has seen their research rapidly translate into a life changing treatment for aHUS patients. It would be a matter of considerable sadness if the benefits of this Kidney Research UK funded translational research would not be available to UK kidney patients.

It seems clear that the expense of Eculizumab is preventing a long term agreement. Kidney Research UK calls on the Department of Health and Alexion pharmaceuticals to engage in a dialog to agree a reasonable price and to end the uncertainty for patients and their families.