NHS England response to:

Evaluation consultation document (ECD2): eculizumab for treating atypical haemolytic uraemic syndrome.

1. NHS England is grateful for the opportunity to comment on this document.
2. We believe that all of the relevant evidence has been taken into account.
3. We believe that the summaries of the criteria considered by the Committee and the clinical and economic considerations are reasonable interpretations of the evidence.
4. We believe that the provisional recommendations are sound and a suitable basis for guidance on the use of eculizumab for the treatment of atypical haemolytic uraemic syndrome in the context of national commissioning by NHS England.
5. We do not think that any aspects of the recommendations need particular consideration to avoid unlawful discrimination against any group of people on the grounds of race, gender, disability, religion or belief, sexual orientation, age, gender reassignment, or pregnancy and maternity.
6. We believe that we can make the health technology available within 3 months, subject to a proviso on the research programme.
7. The Committee has set the following conditions for the use of eculizumab in aHUS:

    - coordination of the use of eculizumab through an expert centre;
    - monitoring systems to record the number of people with a diagnosis of atypical haemolytic uraemic syndrome, the number of people who receive eculizumab, and the dose and duration of treatment for these people
    - a national protocol for starting and stopping eculizumab for clinical reasons
    - a research programme with robust methods to evaluate when stopping treatment or dose adjustment might occur.

    The first three requirements are straightforward to implement. A research programme will take longer to establish. Ideally this should be multi centre and international so as to recruit large numbers of patients: we understand that the UK clinical experts are applying for European funding. If that proposal fails, we will work with the National Institute for Health Research to set up an England-only trial. Agreeing the protocol, securing ethical consent and clearing research governance procedures is likely to take 12 – 18 months.