RESPONSE TO CONSULTATION FROM THE BRITISH ASSOCIATION FOR PAEDIATRIC NEPHROLOGY

Dear Ms Moore

The BAPN was very disappointed to see it is proposed children are excluded from this guidance. Children do get AAV and there is significant morbidity – in one recent publication 3 of 13 children developed end stage renal disease (Siomou et al, Pediatr Nephrol 2012; 27: 1911-20) and 7 were found to have CKD (eGFR <90) on long term follow up. Furthermore, 11 of these children had extra-renal disease. This is consequently a disease that is vigorously treated with cyclophosphamide and intravenous steroids, and many paediatric units also use plasmapheresis to induce remission.

The implications for the use of cytotoxic therapy in childhood are significant because children's life span after treatment is greater than for adults, with consequently more time for effects on fertility and risk of malignancy to be manifest. Therapy that avoids exposure to cytotoxic drugs are likely to be advantageous for children although we acknowledge there is no evidence to support this view.

Plasmapheresis is commonly used as part of induction therapy for children with AAV – consideration should be given to including this therapy in any model of costs.

Section 4.19 considers it is appropriate to exclude children for a purely technical reason – however rituximab is used in children and consequently they should be included in any future appraisal of this technology. Children are disadvantaged by any technology appraisal process that places emphasis on clinical trial data because this is often lacking for valid reasons, including: many diseases are much less common in children making recruitment of an adequate number of participants logistically difficult; clinical trials of therapy for acute illness can be difficult to conduct in children for ethical reasons; pharmaceutical companies are less inclined to support trials in children because the market for products is small. The BAPN would consequently wish a paediatric nephrologist with specialist knowledge of the treatment of AAV to be included in the Guideline Development Group to ensure adequate representation of this important population.

The BAPN is aware that a consortium of professional organisations, including the Renal Association (of which the BAPN is a part) has formulated a response to this consultation. The content of this response has been shared with the BAPN. The BAPN is fully supportive of the submission. Although the data provided pertains to adult patients the use of this therapy in children is guided by experience of its use in adults.

Yours Sincerely

[redacted]