Welsh Kidney Patients Association
Cymdeithas Cleifion Arennau Cymru

[redacted]

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Highly Specialised Technology Evaluation

Eculizumab for the treatment of atypical haemolytic uraemic syndrome (aHUS) [ID703]

Evaluation consultation document

The WKPA would like to submit the following comments on the above consultation. We have three members who suffer with aHus who approached the WKPA to assist their efforts to have Eculizumab approved for their treatment .We have anecdotal evidence that there are approximately 12 patients in Wales and believe that one patient is receiving Eculizumab, having had approval under the Individual Patient Funding Request.
Whilst this consultation is for patients in England, it is hoped that the AWMSG will also approve Eculizumab for treatment of people with aHus in Wales. Should NICE approve its use in England; this will enhance the prospects for Welsh patients to receive this treatment.
We note from the evaluation consultation document that the Committee agrees that treatment with Eculizumab greatly improves the quality and length of life for patients who have aHus. However, the Committee expressed concerns over the high cost of this treatment and stresses the fact that the drug company has not divulged its findings on the long term cost of the treatment. There are also concerns regarding the reasons for the high cost of Eculizumab. We note that the Committee intends to investigate this further.

However, the WKPA would like to draw your attention to the commitments set out in the UK Strategy for Rare Diseases published in November 2013.

In this the four countries of the UK gave the following commitment to sufferers of rare diseases:

- promote equity of access – allowing everyone with a rare disease to follow a clear, well defined care pathway, providing high quality services for every individual through integrated personal care plans

Committee Members:
[redacted]

* offer a patient centred, coordinated approach to treatment services, specialist healthcare and social care support which takes into account the needs of patients, their families and others who provide essential support
* deliver evidence-based diagnosis and treatment of rare diseases, developed through the best use of regional and national resources that are easily accessible by patients and professionals
* support specialised clinical centres to provide expert, high quality clinical care and expertise to patients their families and carers and the patient's, multi-professional healthcare team
* promote excellence in research and develop our understanding of and treatments for rare diseases
* deliver rapid and effective translations of advances in the understanding of rare diseases into clinical care by creating appropriate infrastructure, care pathways and clinical competences
* deliver effective interventions and support to patients and families quickly, equitably and sustainably
* promote collaborative working between the NHS, research communities, academia and industry wherever possible to facilitate better understanding about rare diseases and how they can be best treated
* support education and training programmes that enable health and social care professionals to better identify rare diseases to help deliver faster diagnosis and access to treatment pathways for patients
* promote the UK as a first choice location for research into rare diseases as a leader, partner and collaborator

All of the above shows a clear commitment to providing equity of access to the best possible care and treatment for all sufferers of rare diseases in the UK.

Whilst we recognise the financial restraints in the NHS, the WKPA is of the opinion that Eculizumab provides far better outcomes for patients with aHus than the current standards treatments and request that the Committee takes into account the above commitments when reaching their final decision.

Yours sincerely,

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21st, March 2014.