Access to newly licensed medicines

aHUSUK

Introduction

aHUSUK is a charity and support group for patients with atypical Haemolytic Uremic Syndrome (aHUS), their families and caregivers throughout UK. (See Note 1)

The trustees of aHUSUK are grateful to the Committee for allowing us to express our views. Without an opportunity like this it is difficult for a small voluntary group to get a hearing. Of course we like to think we do not just act for our members but indirectly for everybody with the disease in UK.

Our submission highlights the lack of access in Scotland to eculizumab (trade name Soliris), a recently licensed drug which has been proved to be highly effective in the treatment of aHUS and which we expect soon to become available in England for every patient with a clinical need. When this happens it will create a huge disparity in the way aHUS is treated between patients in England and Scotland. Our submission incorporates the opinions of leading clinicians in this field and the experiences of our members, some of whom have had to cope with the debilitating effects of this disease for more than 15 years.

Background to aHUS

aHUS is a devastating, progressive, life-threatening, incurable blood disorder, which is largely genetic in origin. And it is ultra-rare. In 2010 there were about 170 people diagnosed with the disease in UK, of whom around 140 were resident in England and 20 or so in Scotland. Between 10 and 15 new cases present in England each year. Clinicians in the field say they suspect there could be twice as many again with the disease who have been wrongly diagnosed. Getting a correct diagnosis in the early stages, particularly in children, is often difficult and the trauma surrounding this is a recurring theme in our members’ accounts of their experiences.

aHUS manifests itself when a genetic mutation in one of seven complement proteins is triggered, usually by an external factor. This causes uncontrolled over-activation of the normally well-regulated blood complement system resulting in damage to red blood cells, which in turn leads to renal failure. Although renal problems are most common, neurological, cardiac and gastrointestinal complications can also result.

Triggers have been known to include organ transplantation, pregnancy, hypertension, viral infections such as HIV, flu’ and pneumonia, bacterial infections such as e-coli and administration of certain anti-cancer drugs. Sometimes no trigger is identified.
The disease occurs most frequently in infancy and early childhood but can arise at any time of life. It affects more females than males. Whenever it occurs in life new patients often experience an early and severe reduction in quality of life.

Some carriers of the mutation never develop the disease, but both patients and carriers who are aware they have the mutation have to live with the knowledge that there is a 50% risk of having passed or passing the mutation to their offspring. The disease runs in families. Although the extent of penetration varies in each, in some it has affected several generations.

**Current Treatment**

Until recently the primary treatment was plasma exchange for pre-dialysis patients and dialysis if the recurring episodes progressively reduced kidney function. Plasma exchange is intrusive, requires permanent lines, has several unpleasant side effects, may only be temporarily effective. Nor is it suitable for everybody.

Dialysis is an even greater burden. Risks include infection from extensive related surgery. Side effects include hypertension, seizures, headaches, fatigue and nausea and strict dietary and fluid intake requirements. The above, coupled with an unrelenting regime of several hours of dialysis three or four times a week places severe restraints on social, work and family life.

Kidney transplants have historically had a very low chance of success in patients with aHUS and are not generally done.

**Recent developments in treatment**

Recently a drug has been developed which in trials and limited clinical use has shown that it can control the blood complement's over-reaction and prevent aHUS patients experiencing kidney failure. It may also provide an opportunity for those on dialysis to get on the kidney transplant list. Many with the disease have been denied hope of transplant for years.

The drug is a monoclonal antibody called eculizumab which acts as a complement inhibitor. On starting treatment pre dialysis patients report an immediate, sustained improvement in their condition. Within days kidney function stabilises and within weeks normal life can be resumed. The drug is infused fortnightly in a session lasting 35 minutes. No serious side effects have been reported.

**The present position in Scotland**

Eculizumab is not recommended by SMC for treatment of aHUS. (SMC Advice of 13 2 2012). Although we have found information hard to obtain, of the 20 or so patients with aHUS in Scotland, we are not aware of any receiving the drug through an IPTR. In fact the only patient in Scotland, whom
we know for certain gets eculizumab, is given it free on compassionate grounds by the manufacturers, we think after expiry of a clinical trial.

The present position in England

Eculizumab is manufactured by Alexion Pharma UK and was recently licensed by EMA in Europe and FDA in US for treatment of aHUS. The drug is not widely available in England but its funding is being assessed by AGNSS. Some PCTs have allowed the drug to be prescribed in advance of the AGNSS recommendation. A few other patients are receiving the drug as part of an ongoing clinical trial or on compassionate grounds.

Recognising that the use of eculizumab in the treatment of aHUS falls outside of its normal QALY criteria, last year NICE allowed referral of the drug to the independent Advisory Group for National Specialised Services (AGNSS). Its brief was to investigate and report to ministers on the case for national funding of the drug in England and for creation of an aHUS national research and treatment centre in Newcastle. We understand AGNSS completed its investigations in June 2012.

As at 6 September 2012 we understand that ministers have received AGNSS’s recommendation but have yet to announce their decision. If they rule in favour it will improve dramatically the position of aHUS sufferers in England by removing the “post code lottery” among PCTs making eculizumab freely available to all patients with clinical need including those seeking transplant.

And finally, it will establish a designated national specialised service for treatment of aHUS in Newcastle under Professor Tim Goodship, the leading UK authority on the disease. Access to specialist advice in Newcastle will be available to clinicians throughout England by telemedicine.

We hope that Scottish residents will get access to this facility and that reciprocal funding arrangements will be made between Scotland’s NSD and England’s NSCT. There are precedents for this type of “cross border cooperation” where recognised highly specialist expertise only exists outside Scotland as contained in NSD’s “List of Nationally Funded Services”. However, until SMC changes it outlook and procedures on orphan drugs and realigns it with English practice it is difficult to see how Scottish access to such a specialist service could work effectively. The well-publicised case of the plight of Scottish patients with Paroxysmal Nocturnal Haemoglobinuria (PNH), a disease with similarities to aHUS, being a case in point.

Our conclusions

- In respect of newly licensed medicines for ultra-orphan diseases like aHUS, we propose that SMC formally recognises such conditions in line with definitions stipulated in England by NICE and also recognises the need for a separate policy mechanism to
review ultra-orphan therapies, similar to that presently undertaken by AGNSS. (see Note 2).

- In the case of aHUS, if the Department of Health both approves national funding for eculizumab in England in September 2012 and for a national centre for treatment of the aHUS in Newcastle, the disparity in treatment available to patients resident in England compared to Scotland, which is already evident, will become huge, creating a gross inequality among UK citizens.

- The Scottish Government should investigate the possibility of access for Scottish residents to the proposed national centre for aHUS in Newcastle thus allowing them access to the best advice and treatment in UK.

- NHS Scotland should investigate the opportunities for joint procurement of orphan drugs with the health services in the other three UK nations.

Notes

1. aHUSUK
   Formed in London in September 2011, aHUSUK held its inaugural general meeting in January 2012 and registered as a Charity in England and Wales (no 1145953) in February 2012. Our seven volunteer trustees and fifty-four members in England and Scotland either have aHUS themselves or are related to or caring for someone who has. In its first year aHUSUK has been supported financially by unconditional grants from Newcastle University and Alexion Pharma UK and donations from members.

2. AGNSS
   AGNSS is an NHS Committee. It is to be disbanded by April 2013 as part of the NHS reforms in England. Its responsibilities will be taken over by NICE whose chairman, Professor Sir Michael Rawlins has said that “NICE will be consulting widely with patients, carers, clinicians, commissioners and industry to ensure that it develops a robust and transparent process for making decisions about these highly specialised (that is low volume/very high cost) drugs” Health Minister Lord Howe said that “NICE will wish to build on the decision making framework that AGNSS has developed to ensure that the needs of people with rare conditions are considered”

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