Single technology appraisal (STA)

Eltrombopag for the treatment of chronic idiopathic (immune) thrombocytopenic purpura

Response to the appraisal consultation document by [redacted] of the ITP Support Association

Has all the evidence available to the Appraisal Committee has been appropriately taken into account?

Yes, I believe so this time. The patient voice was heard and the ACD reflects this.

Are the summaries of clinical and cost effectiveness reasonable interpretations of the evidence?

4.17 The Committee was aware that the UK ITP Registry collects data on the long-term outcomes of patients treated with romiplostim and eltrombopag. The Committee concluded that these data would be useful for future appraisals of treatments for chronic ITP. This is an important issue as the medium-tolong-term follow up of patients may resolve the issue of clinical effectiveness between romiplostim and eltrombopag which cannot be accurately assessed from the currently available short-term clinical trials.

The ITP Support Association would like to see mandatory enrolment on the ITP registry for patients on TPO drugs as long term efficacy and safety data is desirable.

Please note that in *Section 4.3 it is stated that "splenectomy would be considered as first-line, second-line or subsequent-line treatment, and that approximately two-thirds of patients can expect splenectomy to succeed. The Committee was aware that splenectomy might be contraindicated in patients at greater risk of bleeding, but that laparoscopic procedures for splenectomy have lowered the risk of bleeding."*

At present there is no clear criteria for clinicians to assess who is at greatest risk of bleeding and other patients types have contraindications for a splenectomy, such as those with asthma; hypertension; cardiac or pulmonary disease; morbid obesity; Common Variable Immune Disease; the elderly; the immunosuppressed; patients who have undergone extensive abdominal surgery; and those who object to losing a healthy organ with less than a two-thirds chance of permanently improving their platelet count. Also, it is important to note that the "two-thirds of patients have their ITP resolved by splenectomy" statement is wholly based on data from the US. It is currently very difficult to assess who will respond and who will not. The indium-labelled spleen scan can be performed to guide a decision on splenectomy, however these scans are not always one hundred percent predictive. Our membership feedback indicates that many are unhappy to lose a healthy organ that may not be implicated in the ITP in the first place. In addition, patients should not undergo splenectomy without first being tested for CVID as the cause of their ITP.

Are the provisional recommendations sound and constitute a suitable basis for guidance to theNHS?

There are potential problems with this provisional Guidance. We have reports from patients (and clinicians) stating that they are unable to access this type of drug without having undergone a splenectomy. We believe that the issue of being 'contraindicated for a splenectomy' requires detailed clarification and no patient should be pressurised into having a splenectomy against their will.

The ITP Support Association urges NICE to include in this guidance the medical, physical, psychological and psychosocial conditions for which splenectomy is contraindicated in ITP. It is unfair to patients and clinicians for decisions to be made on whether a splenectomy is contraindicated without clarity on what exactly falls within this term, and at present this is resulting in a lack of consistency from one hospital to another in England and Wales.

Are there any equality-related issues that need special consideration that are not covered in the ACD?

We already have received feedback from clinicians that they are not being allowed access to (licensed) TPO drugs; this information was mentioned at the Appraisal Committee Meeting. To allow equitable access to these new treatments the guidance from NICE needs to be clear and robust otherwise the budget holders who have no understanding of the condition can choose to sidestep NICE guidance making the whole procedure a total waste of time and money.