The ITP Support Association
Immune thrombocytopenia
itp
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Re: NICE Eltrombopag TA205
Second ACD comments

Has all of the relevant evidence been taken into account?

No.

NICE has not taken into account our comments to the first ACD regarding splenectomy and patient preference not to lose a healthy organ.

Responses to splenectomy are highly variable in people with immune thrombocytopenia (ITP). Patients being asked to have a splenectomy can first check that the spleen is involved in destroying their platelets by having an indium-labelled spleen scan. It is not sensible or acceptable to remove a healthy organ that defends the body against infection as a stepping stone to trying a new drug. This just increases the disease burden of the person involved and may lead to an early death.

With regard to the ITP Registry (para 4.22) we are disappointed by your comment 'these data would be useful for future appraisals' instead of a much needed firm recommendation that all patients on these drugs should be monitored by a specialist haematologist and be enrolled on the ITP Registry for long term follow up.

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

Head-to-head clinical studies are desirable for all ITP treatments but large enough studies are difficult to achieve due to the small patient numbers available.

I participated in the appraisal meeting and am perplexed by your statement (para 4.11) 'The Committee agreed with the clinical specialists that the available evidence suggested that romiplostim was likely to be more effective than eltrombopag rather than equally effective'. I was not aware that the clinical experts stated or implied this at any time, and understood from them that the two drugs were equally effective.

Are the provisional recommendations sound and a suitable basis for guidance to the NHS

The guidance for romiplostim and eltrombopag needs to be similar given that their marketing authorisations are identical (TA221 and TA205).

The indication for romiplostim is as follows (romiplostim FAD TA221 February 2011):

"Romiplostim has a marketing authorisation 'for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)'. The marketing authorisation also states that romiplostim 'may be considered as second line treatment for adult non-splenectomised patients where surgery is contra-indicated'."

From this ACD (TA205 March 2013) for eltrombopag it is stated that:

"Eltrombopag has a UK marketing authorisation for the treatment of adult chronic ITP in patients who have had a splenectomy and whose condition is refractory to other treatments (for example, corticosteroids or intravenous immunoglobulins), and as a second-line treatment for patients who have not had a splenectomy because surgery is contraindicated."

It will be perverse and confusing if NICE guidance for eltrombopag insists on splenectomy where there is no evidence that the spleen is involved. The Association suggested before that the 'contraindications to splenectomy or surgery' were clarified and this has not been done in this draft guidance.

As with romiplostim, eltrombopag should be prescribed and supervised by a haematologist to ensure its appropriate use.

Unlawful discrimination or groups who will be disadvantaged?

NICE, with this draft guidance, puts doctors in an indefensible position and lays the NHS wide open to a legal class action by patients that are forced into a surgical procedure that may not be relevant to, or in fact resolve, their ITP. Splenectomy failure may lead to early death in patients who already have a burden of disease from their ITP and other co-morbid conditions; this is indefensible medical management of people with ITP.