Comments on the ACD Received from the Public through the NICE Website

Name	
Role	NHS Professional
Other role	
Location	England
Conflict	yes
Notes	I run the only tertiary referral centre in the UK for adult ITP. Through this we have developed a UK ITP Registry which now has over 1,000 patients on its database contributed to by many hospitals in the UK. We conduct clinical and research studies and have received funding from many of the companies involved in ITP research. These include companies involved in research in TPO agonists such as Amgen and GSK, with the 2 licensed products, but also Eisai and Shionogi plus companies with other products such as Bayer, BPL, Baxter and Octapharma. I am also involved with the ITP Patient Support Association and am their senior medical advisor.
Comments on individual sections of the ACD:	
Section 1 (Appraisal Committee's preliminary recommendations)	There are two licensed products on the market and the licensed indications are identical. The recommendations for Romiplostin (TA 221) differ significantly from those suggested here. These suggest: Romiplostim is recommended for the treatment of adults with chronic immune (idiopathic) thrombocytopenia purpura whose condition is refractory to standard active treatments and rescue therapies or who have severe disease and a high risk of bleeding that needs frequent courses of rescue therapies and if the manufacturer makes romiplostim available with the rebate on the list price agreed under the patient access scheme. Only a haematologist should start and supervise treatment with romiplostim. By having such different indications it suggests that these products differ in some way and this could potentially lead clinicians to consider that they should be used for different clinical indications and in different patient populations. The clinical evidence does not support this.
Section 2 (The technology)	
Section 3 (The manufacturer's submission)	
Section 4 (Consideration of the evidence)	
Section 5 (Implementation)	In the preliminary recommendations for Romiplostim NICE suggested that there should be a register to record usage and outcome. This did not appear in the final recommendations although it had quite widespread clinical support. In addition to providing important information on clinical outcome in 'real-life' patient populations rather than the more restricted groups in the trials this would have recorded important information on side-

	effects. While there is little to suggest that there is an increased risk of malignancy this remains a theoretical risk from these agents effect on stem cells. We know from the results with erythropoietin that this impact may be delayed for some years. While post-marketing surveillance is important this would have provided some rigour to the follow-up. I do however accept that having not imposed this on Romiplostim it would be unfair to expect it with Eltrombopag, however, it may give the collection of data some weight if this was emphasised.
Section 6 (Proposed recommendations for further research)	This is difficult as most of the agents used in the treatment of ITP are unlicensed for this condition. For the most promising agent Roche have refused to support studies and the meta-anlyses suggest that despite early responses the long term results are no better than standard therapy. However, many in the community are keen to develop 'own account' studies and such a recommendation will help the development of good quality collaborative studies.
Section 7 (Related NICE guidance)	Referring back to my comments on recommendations the insertion of the requirement to use Eltrombopag post-splenectomy is not in accord with the recommendations for splenectomy. The clinical results for both agents are similar and although the drugs have slightly different characters (subcut v oral) they are essentially interchangeable. As the cost effectiveness is reflected in the saving of IV immunoglobulin usage the use in refractory patients, or those at risk from bleeding should be emphasised and the splenectomy failure down-played. As there is now increasing evidence that outcome of splenectomy can be predicted using Indium-labelled platelet studies it is not sensible to insist on this, but unfortunately many commissioners have seized on this aspect and in consequence many patients have been subject to irrelevant surgery. The recommendation that the drug should be given under the control of a specialist haematologist should also be emphasised. These drugs are tricky to administer and should not be used by those not experienced in the disease. This will also aid the collection of long term outcome data. I hope that these two recommendations can be reconciled.
Section 8 (Proposed date of review of guidance)	
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