Romiplostim for the treatment of chronic immune or idiopathic thrombocytopenic purpura

Appraisal consultation document – commentator comments

Headings for response	Response from GSK
Do you consider that all the relevant evidence has been taken into account?	**ACD 3.20 p. 12** *'The ERG noted that limited evidence was available on the treatment of patients with chronic ITP, including with romiplostim and potential comparators, and particularly for long-term outcomes. The ERG also considered that, for comparators, evidence for non-splenectomised and splenectomised patients was commonly not distinguished.'* GSK supports the ERG's conclusion that there is limited evidence available for comparative treatments and their long-term outcomes. Further, there is a poor body of evidence to support comparative efficacy by splenectomy status. The heterogenic nature of the available evidence makes synthesis or pooling of the data particularly challenging but is not a reason to discount such approaches. **ACD 4.7 p. 17** *'The Committee concluded that appropriate comparator pathways for romiplostim are those that start with active treatments and use 'watch and rescue' alongside these rather than the comparator pathway in the manufacturer's base case, which commenced with 'watch and rescue' alone.'* GSK understands, from advisory board discussions with ITP experts across the UK, that a watch and rescue treatment strategy is applicable to a chronic ITP patient population. A watch and rescue management strategy encapsulates a broad spectrum of chronic ITP patients including those that may have responded inadequately to active treatments. All watch and rescue patients are actively monitored by their clinician. Some patients in this category require only rescue

	medication as necessary (e.g IV Ig). However other watch and rescue patients require ongoing pharmacological intervention with non-selective immunosuppressive agents and when necessary require additional rescue medication. In this setting, non-selective immunosuppressive agents include but are not limited to: steroids (e.g. prednisolone); immunosuppressants (e.g. ciclosporin); endocrine drugs (e.g. danazol); and cytotoxic drugs (e.g. vincristine).
Do you consider that the summaries of the clinical and cost effectiveness are reasonable interpretations of the evidence, and that the preliminary views on resource impact and implications for the NHS are appropriate?	No comment.
Do you consider that the provisional recommendations of the Appraisal Committee are sound and constitute a suitable basis for the preparation of guidance to the NHS?	No comment.
Are there any equality related issues that need special consideration that are not covered in the ACD?	No comment.