1. *Do you consider that all of the relevant evidence has been taken into account?*

No, the ITP Support Association believes that immune (idiopathic) thrombocytopaenic purpura (ITP) has been misunderstood.

1.1 **ITP is a rare condition with life-threatening consequences for some people**

The ITP Support Association does not believe that the Committee has truly understood the nature of the condition. It appears that NICE believe ITP to be a condition that has 'lifestyle consequences only'. However, we have members of our Association who have regular bleeds during normal daily activities, some of which are life-threatening.

We are aware of some patients/members of the ITP Support Association who have life-threatening ITP, which is resistant to all current treatment. Obviously, none of these patients were available to attend the NICE Appraisal Committee meeting as their current condition means that they are housebound and unable to travel due to frequent bleeds. There are patients who have died due to major bleeds in spite of clinicians attempts to prevent this; these patients may still be alive today if romiplostin had been available as a treatment option.

1.2 **Important unmet medical needs exist**

ITP is a heterogenous disease, afflicting patients in very different ways, some ITP patients are able to lead a relatively normal life, however, other patients will have dangerously low platelet counts and experience symptomatic bleeding, which in severe cases will be fatal. It is vital that NICE recognises the fact that patients need to be offered a tailored approach with respect to the management for their ITP. The recent International Consensus on the investigation and management of primary immune thrombocytopenia clearly illustrates the need for a tailored treatment approach as all treatment options are listed alphabetically so as to show no preference for a particular therapy. This has been pre-published on-line see Blood Oct 2009, DOI 10.1182.

It is acknowledged by clinicians who advise the UK ITP Support Association that there is still a great unmet need in terms of managing patients with severe ITP who are refractory to other treatment options (including splenectomy) whereby the life-threatening bleeding risk remains high. The ITP Support Association strongly urges NICE to acknowledge this unmet need as the Scottish Medicines Consortium (SMC) and the The European Medicines Agency (EMEA) have.

1.3 **A potentially life-threatening condition with limited treatment options**

NICE has commented that there are no comparative trials of romiplostim versus any of the current second-line treatment options for ITP, however it is widely acknowledged that a wide range of treatment options are used to

treat patients with ITP and that many of these treatment options are unlicensed, which would therefore make it difficult and possibly unethical to conduct comparative trials with romiplostim.

The ITP Support Association draw NICE's attention to the EMEA report on romiplostin (p.59), which notes that 'The benefits of romiplostin in terms of platelet count have been demonstrated and replicated in two independent randomised clinical trials. This **strength of evidence is uncommon in an orphan condition such as ITP and the effect of romiplostim should be placed in the context of a life-threatening disease where limited therapeutic alternatives are available'** (The European Medicines Agency – EMEA – European Public Assessment Report, Romiplostin [Nplate]. 16/2/2009, EMEA H-C-942-00-00. www.emea,europa.eu).

2. Do you consider that the summaries of clinical and cost effectiveness are reasonable interpretations of the evidence, and that the preliminary views on the resource implications for the NHS are appropriate?

No, the ITP Support Association believes that the interpretation of the evidence are unreasonable for a rare and sometimes, life-threatening condition with few effective treatment options.

2.1 Clinical effectiveness; better evidence than many of the other treatment options?

Although the ITP Support Association recognises that this has been a thorough review, there is considerable discomfort that this review fails to acknowledge the clinical effectiveness of romiplostin, which is based upon some of the largest randomised controlled trials in patients with ITP. Many of the other treatments currently in use do not have this level of evidence of their effectiveness and many are not licensed for this indication.

2.2 Cost effectiveness; accurate assessment of the patient cohort that benefits the most?

The Scottish Medicines Consortium (SMC) report recognises that given the potentially life-saving nature of this orphan drug, the economic case for use can be made. The SMC note that the Incremental Cost Effectiveness Ratio (ICER) is acceptable when focusing on a sub group of patients for whom the savings in terms of rescue medications are more probable i.e those with severe clinical signs or those people most at risk of bleeding. We ask NICE to consider the cost effectiveness of romiplostin further.

2.3 Resource implications; practice shows that a small patient population benefit the most

Our clinical experts who are currently working with romiplostin believe that 'in practice it is a small population of patients who will receive this treatment'. This is reflected in the comments in the SMC review and in the EMEA appraisal of the product. Both recognise that the life-threatening

nature of the condition endured by some patients demands that romiplostin is available as a treatment option.

3. *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, the ITP Support Association does not, although the ITP Support Association agrees with NICE (section 4.13 in the Appraisal Consultation Document) that, "In summary, the Committee considered that, in current UK practice, treatment with romiplostim would be prescribed only for people with chronic ITP who have symptoms or a high risk of bleeding severe enough to warrant intervention".

3.1 Lack of recognition of orphan status, which has been recognised by the Scottish Medicines Consortium (SMC)

The SMC now recommend the restricted use of romiplostim in NHS Scotland for adult chronic ITP splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Romiplostim is also accepted for restricted use as a second line treatment for adult non-splenectomised patients where surgery is contra-indicated. Romiplostim is restricted to use in patients with severe symptomatic ITP or patients with a high risk of bleeding.

3.2 What about the cohort with life threatening issues?

The ITP Support Association strongly urges NICE to appreciate the fact that there is a small cohort of patients with severe ITP who are at a high risk of life-threatening bleeding who will benefit from treatment with romiplostim. We urge NICE to make this treatment available for this group of patients with the greatest unmet need.

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

4.1 Equitable access across the regions of the UK

The ITP Support Association would like to NICE reach the same conclusion as the Scottish Medicines Consortium. This ensures that **ITP patients in England/Wales and Northern Ireland receive equitable access to romiplostim.**