Note: Confidential information is redacted

27 October 2010

Dr Elisabeth George
Associate Director - Appraisals
National Institute for Health and Clinical Excellence
London WC1V 6NA
United Kingdom

Dear Elisabeth

We are writing, in confidence, to highlight forthcoming changes to the romiplostim summary of product characteristics (SPC) with regards to dose adjustments related to platelet counts that are relevant for the upcoming Appraisal Committee (AC) meeting to discuss romiplostim on Thursday 4th November 2010. [redacted]

[redacted] Our economic model assumed that romiplostim would be dosed in clinical practice as it was in our pivotal phase III trials. The ERG in their report (commenting on our patient access scheme submission) state that, *"It should be noted that this uncertainty may also mean that the manufacturer's data overestimate routine NHS dosages. In such a situation, other things being equal, the cost-effectiveness of romiplostim will improve."* [redacted]

Therefore, romiplostim is likely to be more cost-effective in clinical practice than estimated in our economic model.

[redacted]

Therefore, the average romiplostim dose observed in our pivotal phase III trials is likely to exceed that of clinical practice. [redacted], a larger proportion of patients would have had their doses withheld than the estimated 5% of the patients who had their dose withheld in the pivotal phase III trials.

[redacted]

[redacted], we would like to bring to the Institutes attention a paper that has been published recently – *Gernsheimer TB, George JN, Aledort LM, Tarantino MD et al. Evaluation of bleeding and thrombotic events during long-term use of romiplostim in patients with chronic immune thrombocytopenia (ITP). J Thromb Haemost 2010; 8:1372–82*. This paper includes information on patient characteristics in terms of bleeding symptoms at the time of enrollment into our phase III trials, e.g. 54% of patients experienced oral bleeding, 50% of all women had menorrhagia, 20% experienced gastrointestinal bleeding, and 6% had an intracranial bleeding. This demonstrates that the patients enrolled into our trials were a group of patients with severe symptomatic ITP and were at a high risk of bleeding.

Yours sincerely

[redacted]

[redacted]
Corporate Affairs