

## FOOD AND DRUG ADMINISTRATION

Center for Drug Evaluation and Research

*Oncologic Drugs Advisory Committee*

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*On May 30, 2008, the committee will discuss new drug application (NDA) 022-291, proposed trade name PROMACTA (eltrombopag olamine), GlaxoSmithKline, proposed indication for the short-term treatment of previously-treated patients with chronic idiopathic thrombocytopenic purpura (ITP) to increase platelet counts and reduce or prevent bleeding..*

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### **Eltrombopag (Promacta®) NDA 22-291 Questions**

In two randomized, "short term" clinical studies of adult patients with chronic immune (idiopathic) thrombocytopenic purpura (ITP), a greater proportion of patients who received eltrombopag experienced a "platelet response" than patients who received a placebo (70% versus 11% in one study and 58% versus 16% in another study). To assess a treatment effect upon bleeding outcomes, the studies used a bleeding scoring system of unclear clinical meaningfulness. The studies signaled risks for serious hemorrhage following the discontinuation of eltrombopag as well as a risk for hepatotoxicity during the drug therapy. Clinical studies intended to thoroughly assess the safety and efficacy of long term eltrombopag use are ongoing and only limited, interim data are available.

1. (Vote) Eltrombopag is proposed for use in patients, such as those undergoing a surgical procedure, who have a specific need for short term therapy. The patients in the completed, controlled studies did not have this specific need and some experienced serious hemorrhage when eltrombopag was discontinued. Since ITP is generally a chronic condition, long term therapy is anticipated. Given these observations, should FDA delay marketing authorization until it has reviewed the final data from the on-going clinical studies (RAISE, EXTEND)?

If no, please answer the next question.

2. (Vote) Do the current clinical data demonstrate a favorable risk-benefit profile for the use of eltrombopag in the "short term" treatment of patients with chronic ITP?