NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal

Eltrombopag for the treatment of chronic idiopathic (immune) thrombocytopenic purpura (review of technology appraisal 205)

Draft scope

Appraisal objective/Remit

To appraise the clinical and cost effectiveness of eltrombopag within its licensed indication for the treatment of refractory chronic idiopathic (immune) thrombocytopenic purpura.

Background

Idiopathic (immune) thrombocytopenic purpura (ITP) is an autoimmune condition characterised by increased platelet destruction and, in some cases, inadequate platelet production. The condition can result in low platelet counts and bleeding. In a blood test, a normal platelet count (concentration) is between 150 and 400 × 10^9 per litre. Bleeding does not usually occur until the platelet count is below 30 × 10^9 per litre. ITP is defined as chronic when it lasts longer than 12 months.

The UK incidence of adult ITP is estimated to be around 120 per year and 3000–3500 people are affected at any one time England and Wales. People with ITP maybe asymptomatic or have symptoms including spontaneous bruising, mucosal bleeding and, in severe cases, gastrointestinal or intracranial bleeding. Diagnosis is based on excluding other possible causes of thrombocytopenia.

Treatment is usually required only when the platelet count is below 30 × 10^9 per litre unless procedures involving blood loss are planned (British Society for Haematology guideline). Treatment is typically initiated with 'rescue therapies', such as corticosteroids and intravenous immunoglobulins, and thereafter with 'active treatments' including splenectomy, rituximab and other immunosuppressive agents. NICE technology appraisal TA221 recommends romiplostim for the treatment of adults with chronic ITP whose condition is refractory to standard active treatment and rescue therapies, or who have severe disease and a high risk of bleeding that needs frequent courses of rescue therapies.

Current NICE guidance TA205 does not recommend eltrombopag within its licensed indication for the treatment of refractory chronic idiopathic (immune) thrombocytopenic purpura in splenectomised adults whose condition is refractory to other treatment (for example corticosteroids, immunoglobins) or as a second-line treatment in non-splenectomised adults where surgery is contraindicated.

The technology

Eltrombopag (Revolade, GlaxoSmithKline UK) increases platelet production through activation of the thrombopoietin receptor. By stimulating platelet production, it helps to reduce bleeding. Eltrombopag is administered orally.

Eltrombopag has a UK marketing authorisation for the treatment of chronic ITP in splenectomised adult patients whose condition is refractory to other treatment (such as corticosteroids and immunoglobulins) and as a second line treatment for non-splenectomised adult patients where surgery is contraindicated.

Intervention	Eltrombopag
Population	Adults with idiopathic thrombocytopenic purpura
Comparators	• corticosteroids • intravenous normal immunoglobulin • immunosuppressive agents including rituximab • romiplostim • intravenous anti-D immunoglobulin (for people where splenectomy is contraindicated)
Outcomes	The outcome measures to be considered include: • platelet count • response rate • duration of response • need for rescue treatments • use of concurrent treatments • reduction in symptoms (minor and/or severe) • mortality • adverse effects of treatment • health-related quality of life

Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The time horizon for the economic evaluation will be based on the appropriate time period over which costs and benefits can reasonably be expected to be experienced given the chronic nature of the condition. The analyses should consider the comparison of treatment sequences with and without eltrombopag, and the frequency of rescue therapies. The analyses must specify if eltrombopag is an addition to, or a replacement of an existing element in, the treatment pathway. Costs will be considered from an NHS and Personal Social Services perspective.
Other considerations	Those patients who have undergone splenectomy will not be offered treatment with anti-D immunoglobulin. Therefore a separate consideration of the pathway of care, clinical and cost effectiveness is appropriate for this subgroup of patients. If the evidence allows, other subgroups may be identified for whom the technology may be particularly clinically and cost effective. Guidance will only be issued in accordance with the marketing authorisation.
Related NICE recommendations	Related Technology Appraisals: Technology Appraisal No. 221, April 2011, 'Romiplostim for the treatment of chronic idiopathic (immune) thrombocytopenic purpura'. Review date March 2014. Technology Appraisal No. 205, October 2010, 'Eltrombopag for the treatment of chronic idiopathic (immune) thrombocytopenic purpura'. Review date TBC.

Questions for consultation

Have the most appropriate comparators for eltrombopag for the treatment of chronic idiopathic (immune) thrombocytopenic purpura been included in the scope? Are the comparators listed routinely used in clinical practice?

Under what circumstances is intravenous anti-D immunoglobulin used for the treatment of chronic idiopathic (immune) thrombocytopenic purpura?

Can a standard sequence of treatments be defined? Is the subgroup suggested in 'other considerations' appropriate? Are there any other subgroups of people in whom the technology is expected to be more clinically effective and cost effective or other groups that should be examined separately?

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which eltrombopag is licensed;

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the Committee to identify and consider such impacts.

Do you consider the technology to be innovative in its potential to make a significant and substantial impact on health-related benefits and how it might improve the way that current need is met (is this a 'step-change' in the management of the condition)?

Do you consider that the use of the technology can result in any potential significant and substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the Appraisal Committee to take account of these benefits

NICE intends to appraise this technology through its Single Technology Appraisal (STA) Process.