NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal

Eltrombopag for the treatment of chronic immune (idiopathic) thrombocytopenic purpura (review of technology appraisal 221)

Final 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

Immune (idiopathic) 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 ITP in splenectomised adults whose condition is refractory to other treatment (for example corticosteroids, immunoglobulin) 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 immune (idiopathic) thrombocytopenic purpura, who • have had a splenectomy and are refractory to other treatments (e.g. corticosteroids, immunoglobulins) • who not have had a splenectomy and for whom surgery is contraindicated, as second line treatment
Comparators	• corticosteroids • intravenous normal immunoglobulin • immunosuppressive agents including rituximab • romiplostim
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	Consideration will be given to subgroups of patients who have • undergone splenectomy • not undergone splenectomy where surgery is contraindicated. 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.