NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Health Technology Appraisal

Romiplostim for the treatment of chronic idiopathic (immune) thrombocytopenic purpura

Final scope

Remit/appraisal objective

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

Background

Idiopathic thrombocytopenic purpura ([ITP] also known as immune thrombocytopenic purpura) is an autoimmune bleeding disorder characterised by increased platelet destruction and additionally, in some cases, inadequate platelet production. With ITP, platelet counts are below 150,000 per microlitre. Counts of 150,000 to 400,000 per microlitre are considered normal in adults. ITP that lasts longer than 6 months is defined as chronic.

The British Society for Haematology (BSH) estimates that the UK incidence of new cases of adult ITP is around 120 per year and 3000–3500 patients are affected at any one time in England and Wales. Individuals 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 30,000 per microlitre unless procedures involving blood loss are planned (BSH guideline). Existing first-line treatments include corticosteroids and intravenous immunoglobulins. It is estimated that 11-35% cases of chronic ITP are refractory to first and second-line therapies. Further treatment options include rituximab, splenectomy and other immune suppressive agents.

The technology

Romiplostim (Amgen) is a protein that increases the production of platelets by stimulating the differentiation and proliferation of megakaryocytes. It is administered by subcutaneous injection.

Romiplostim has been studied in patients with ITP (splenectomised and non-splenectomised) with platelet counts less than 30,000 per microlitre and in whom at least one prior treatment regimen has failed

Intervention(s)	Romiplostim
Population(s)	Adults with idiopathic thrombocytopenic purpura with platelet counts less than 30,000 per microlitre in whom at least one prior treatment regimen has failed.
Standard comparators	People who have not had a splenectomy: • corticosteroids • intravenous normal immunoglobulin • intravenous anti-D immunoglobulin • rituximab • splenectomy • immunosuppressive agents People who have had a splenectomy: • corticosteroids • intravenous normal immunoglobulin • rituximab • immunosuppressive agents
Outcomes	The outcome measures to be considered include: • platelet count • response rate • durable response • need for rescue treatments • use of concurrent treatments • reduction in symptoms (minor and/or severe) • adverse effects of treatment • mortality • 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 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. 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 Appraisal (in progress): Eltrombopag for the treatment of chronic idiopathic (immune) thrombocytopenic purpura. Earliest anticipated publication: TBC