# NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

## Health Technology Appraisal

## AMG 531 for the treatment of chronic immune (idiopathic) thrombocytopenic purpura

## Draft scope (Pre-referral)

### Draft remit/appraisal objective

To appraise the clinical and cost effectiveness of AMG 531 for the treatment of refractory chronic immune (idiopathic) thrombocytopenic purpura.

### Background

Immune thrombocytopenic purpura (also known as idiopathic thrombocytopenic purpura [ITP]) is an autoimmune bleeding disorder characterised by increased platelet destruction and/or inadequate platelet production. With ITP, platelet counts are below 150,000/µl. Counts of 150,000 to 400,000/µl are considered normal in adults. ITP that lasts longer than 6 months is defined as chronic.

The British Haematology Society estimates that the UK incidence of new cases of ITP is around 5.8 – 6.6 per 100,000 population per year (equivalent to 3,000 – 3,500 patients in England and Wales). ITP is three times more common in women than in men. 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 <30,000/µl unless procedures involving blood loss are planned (British Haematology Society guideline). Existing first-line treatments include corticosteroids and intravenous immunoglobulins. Second-line options include; splenectomy, high dose corticosteroids, high dose normal immunoglobulin, intravenous anti-D ($Rh_0$) immunoglobulin, vinca alkaloids, immunosuppressive agents, danazol and dapsone. It is estimated that 11-35% cases of chronic ITP are refractory to first and second-line therapies. Third-line treatment options are limited and include drugs such as rituximab, alemtuzumab and mycophenolate mofetil.

### The technology

AMG 531 (Amgen) is a protein that increases the production of platelets by stimulating the differentiation and proliferation of megakaryocytes. It is administered by subcutaneous injection.

AMG 531 has been studied in patients with ITP with platelet counts less than 30,000/µl and in whom at least one prior treatment regimen had failed

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| **Intervention(s)** | AMG 531 |
| **Population(s)** | Adults with idiopathic thrombocytopenic purpura with platelet counts less than 30,000/µl in whom at least one prior treatment regimen has failed. |
| **Standard comparators** | • Alemtuzumab<br>• Danazol<br>• Dapsone<br>• Immunosuppressive agents<br>• Mycophenolate mofetil<br>• Rituximab<br>• Splenectomy<br>• Vinca alkaloids |
| **Outcomes** | The outcome measures to be considered include:<br>• platelet count<br>• response rate<br>• duration of response<br>• need for rescue treatments<br>• reduction in symptoms (minor and/or severe)<br>• adverse effects of treatment<br>• mortality<br>• 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.<br>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<br>Costs will be considered from an NHS and Personal Social Services perspective. |
| **Other considerations** | If the evidence allows, subgroups may be identified for whom the technology may be particularly clinically and cost effective.<br>Guidance will only be issued in accordance with the marketing authorisation. |

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| **Related NICE recommendations** | There are no related Technology Appraisals or Guidelines |

## Questions for consultation

Are the suggested comparators appropriate?

Should this technology be appraised through the single technology appraisal process or would it be more appropriate to appraise AMG 531 together with eltrombopag in a multiple technology appraisal. (Further information on these processes is available from URL http://www.nice.org.uk/page.aspx?o=taprocess)

How is response defined in this condition?

Are there any groups of patients in which AMG 531 would be expected to be more or less clinically effective or cost effective or groups that should be examined separately?