

**EMBARGOED UNTIL: 00:01 BST Wednesday 27 April 2011**

## **News Release**

### **NICE recommends romiplostim for the treatment of rare blood disorder**

In final guidance issued today (Wednesday 27 April 2011), the National Institute for Health and Clinical Excellence (NICE) has recommended romiplostim (Nplate, Amgen) for the treatment of patients with severe, chronic immune (idiopathic<sup>1</sup>) thrombocytopenic purpura (ITP).

ITP is a rare bleeding disorder in which the immune system destroys platelets, which are needed for normal blood clotting. People with the disease have abnormally low levels of platelets in the blood. The condition is currently thought to affect 3000 – 3500 people in the UK; some patients may only have the condition for a short period of time, but when a patient has ITP for over 12 months this is defined as having chronic ITP.

In this final guidance, romiplostim is recommended for the treatment of adults with chronic ITP:

- whose condition does not respond to standard active treatments and rescue therapies **or**,
- who have severe disease, and a high risk of bleeding that requires frequent courses of rescue therapies, **and**
- if the manufacturer makes romiplostim available with the rebate on the list price agreed under the patient access scheme<sup>2</sup>.

Only a haematologist should initiate and supervise treatment with romiplostim.

**Dr Carole Longson, Director, Health Technology Evaluation Centre at NICE**

**said:** “We are pleased to be able to recommend the use of romiplostim as a clinically and cost effective treatment for some people with severe, chronic ITP, a serious and sometimes debilitating disorder. The manufacturer submitted a patient access scheme, and the cost of the treatment to the NHS is reduced.”

The **final guidance** is available from **Wednesday 27 April** on the NICE website at <http://nice.org.uk/guidance/TA221>

Once NICE issues its final guidance on a technology, it replaces local recommendations across the country.

For more information, please call the NICE press office on 0845 003 7782 or out of hours on 07775 583 813.

**Ends**

**Notes to Editors**

**About the guidance**

1. The **final guidance** is available from **Wednesday 27 April** on the NICE website at <http://nice.org.uk/guidance/TA221>
2. Romiplostim (Nplate, Amgen) is a protein that mimics the action of thrombopoietin (a glycoprotein hormone produced mainly by the liver and the kidneys that regulates the production of platelets by the bone marrow) by acting as an agonist at thrombopoietin receptors. It stimulates the differentiation and proliferation of bone marrow cells responsible for producing platelets (megakaryocytes), and so increases platelet production and platelet counts.
3. The summary of product characteristics (SPC) states that the recommended initial dose of romiplostim is 1 microgram/kg of actual body weight, administered once weekly as a subcutaneous injection. The dose may be adjusted by increments of 1 microgram/kg until a platelet count equal to or above  $50 \times 10^9$  platelets per litre of blood is reached. A maximum dose of 10 micrograms/kg once weekly should not be exceeded. Platelet counts should be measured weekly until a stable count equal to or above  $50 \times 10^9$  platelets per litre for at least 4 weeks without adjusting the dose. Thereafter, platelet counts should be measured monthly. Treatment with romiplostim should be stopped if the platelet count does not increase sufficiently to avoid clinically significant bleeding after 4 weeks of romiplostim therapy at the highest weekly dose of 10 micrograms/kg. Romiplostim should also be stopped if a peripheral blood smear indicates increased bone marrow reticulin (collagen-like protein fibres found in tissue) as well as if a loss of efficacy is observed.
4. The list price of romiplostim is £1.93 per microgram; therefore a 250 microgram vial costs £482 (excluding VAT; British national formulary [BNF] edition 60). The cost of treatment varies depending on the patient’s weight, the dosing regimen and any waste that results from discarding any unused drug from the single use of a 250 microgram vial. Based on this list price the annual cost of romiplostim treatment for a person weighing 80 kg would be £8020 at a dose of 1 microgram/kg weekly and £80,204 at a dose of 10 micrograms/kg weekly (assuming no waste).

5. The manufacturer of romiplostim (Amgen) has agreed a patient access scheme with the Department of Health which makes romiplostim available with a discount on the 250 microgram vial. The size of the rebate is commercial in confidence. The Department of Health considered that this patient access scheme does not constitute an excessive administrative burden on the NHS. It is the responsibility of the manufacturer to communicate details of the discount to the relevant NHS organisations. Any enquiries from NHS organisations about the patient access scheme should be directed to the manufacturer at the following e-mail address:  
[nplate-nicepas@amgen.com](mailto:nplate-nicepas@amgen.com)
6. The manufacturer has agreed that the patient access scheme will remain in place until any review of this NICE technology appraisal guidance is published.
7. There is further information on patient access schemes on the NICE website at:  
<http://www.nice.org.uk/aboutnice/howwework/paslu/patientaccessschemesliaisonunit.jsp>
8. ITP is more common in women. Among both women and men, incidence is higher in older people.
9. In adults, ITP comes on gradually and it usually does not follow a viral illness. There may be no symptoms, mild bruising or bleeding, or severe bleeding.
10. Because most adults with ITP do not have any symptoms, ITP is usually diagnosed on a routine blood test that has been done for other reasons. The full blood count shows a lower number of platelets than normal.
11. The NICE guidance on eltrombopag (Revolade, GlaxoSmithKline) for chronic immune (idiopathic) ITP can be found at <http://guidance.nice.org.uk/TA/Wave17/14>

## References

1. Of unknown cause.
2. Patient access schemes are ways pharmaceutical companies can propose to enable patients to gain access to high cost drugs.

## About NICE

1. The National Institute for Health and Clinical Excellence (NICE) is the independent organisation responsible for providing national guidance and standards on the promotion of good health and the prevention and treatment of ill health.
2. NICE produces guidance in three areas of health:
  - **public health** – guidance on the promotion of good health and the prevention of ill health for those working in the NHS, local authorities and the wider public and voluntary sector
  - **health technologies** – guidance on the use of new and existing medicines, treatments, medical technologies (including devices and diagnostics) and procedures within the NHS
  - **clinical practice** – guidance on the appropriate treatment and care of people with specific diseases and conditions within the NHS.
3. NICE produces standards for patient care:

- **quality standards** – these reflect the very best in high quality patient care, to help healthcare practitioners and commissioners of care deliver excellent services
- **Quality and Outcomes Framework** – NICE develops the clinical and health improvement indicators in the QOF, the Department of Health scheme which rewards GPs for how well they care for patients.

4. NICE provides advice and support on putting NICE guidance and standards into practice through its **implementation programme**, and it collates and accredits high quality health guidance, research and information to help health professionals deliver the best patient care through **NHS Evidence**.