National Institute for
Health and Clinical Excellence

Tel: 0845 003 7782
www.nice.org.uk
Ref: 2010/170

Press Release

NICE recommends romiplostim for the treatment of chronic idiopathic thrombocytopenic purpura (ITP)

In preliminary draft guidance issued today (Wednesday 1 December 2010) by the National Institute for Health and Clinical Excellence (NICE), romiplostim (Nplate, Amgen) is recommended for the treatment of chronic idiopathic (immune) thrombocytopenic purpura (ITP) in some patients.

ITP is a bleeding disorder caused by 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 6 months this is defined as having chronic ITP.

In this draft guidance, romiplostim is recommended for the treatment of some adults with chronic ITP:

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

Only a specialist in haematology should initiate and supervise treatment with romiplostim.

This draft guidance has been issued for consultation; NICE has not yet issued final guidance to the NHS.

Dr Carole Longson, Director, Health Technology Evaluation Centre at NICE said: "We are pleased to recommend the use of romiplostim as a clinically and cost effective treatment for some people with severe, chronic ITP in our draft guidance issued today. ITP is a serious, often debilitating disorder, and some of the current treatments have considerable side effects, so this will be welcome news to all those affected."

The **draft guidance** is available for public consultation on the NICE website until Tuesday 11 January 2011 at http://guidance.nice.org.uk/TA/Wave17/16

Comments received during this consultation will be reviewed by the independent Appraisal Committee at their next meeting on Thursday 3 February. NICE expects to issue final guidance to the NHS in 2011. Until this time, NHS bodies should make decisions locally on the funding of specific treatments. Once NICE issues its final guidance on a technology, it replaces local recommendations across the country.

Final guidance is likely to be published in 2011.

Ends.

Notes to Editors

About the guidance

1. The **draft guidance** is available for public consultation on the NICE website until Tuesday 11 January 2011 at http://guidance.nice.org.uk/TA/Wave17/16
2. Romiplostim (Nplate, Amgen) is a protein that mimics the action of thrombopoietin (a glycoprotein hormone produced mainly by the liver and the kidney that regulates the production of platelets by 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 50×10^9 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 50×10^9 platelets per litre for at least 4 weeks without dose adjustment is observed. Thereafter, platelet counts should be measured monthly. Treatment with romiplostim should be discontinued 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 discontinued if a peripheral blood smear indicates increased bone marrow reticulin.
4. Romiplostim costs £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. 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). The manufacturer of romiplostim (Amgen) has agreed a patient access scheme with the Department of Health which offers a rebate on the list price of the 250 microgram vial of romiplostim. The size of the rebate is commercial in confidence.

5. About 24 per 100,000 adults have ITP. It is more common in women. Among both women and men, incidence is higher in older people.
6. 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.
7. 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.
8. The NICE guidance on eltrombopag (Revolade, GlaxoSmithKline) for chronic immune (idiopathic) ITP can be found at http://guidance.nice.org.uk/TA/Wave17/14

About NICE

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. NICE produces guidance in three areas of health:

1. public health
2. health technologies, and
3. clinical practice

NICE also produces standards for patient care through its work on the Quality and Outcomes Framework and quality standards. With high quality health information collated through NHS Evidence, all of NICE's work is supported by its implementation programme.