ITEM 5

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Technology Appraisals

Consultation on Batch 16 draft remits and draft scopes

Summary of comments and discussions at scoping workshops

	Batch 16 topics
5.1	Pegaptanib sodium for the treatment of diabetic macular oedema
5.2	Plerixafor for haematopoietic stem cell mobilisation prior to autologous stem cell transplantation in the treatment of lymphoma
5.3	Plerixafor for haematopoietic stem cell mobilisation prior to autologous stem cell transplantation in the treatment of multiple myeloma
5.4	Prolonged-release fampridine for the improvement of walking ability in multiple sclerosis. *The decision on the referral of prolonged release fampridine for the improvement of walking ability in multiple sclerosis has been deferred until the outcome of the regulatory process is known.*
5.5	Duloxetine for the treatment of chronic somatic pain
5.6	Percutaneous vertebroplasty and percutaneous kyphoplasty for the treatment of osteoporotic vertebral fractures
5.7	Adalimumab for the treatment of juvenile idiopathic arthritis *(re-scoping to cover an application for a licence extension, not part of Batch 16)*

Provisional Title	Pegaptanib sodium for the treatment of diabetic macular oedema
Topic Selection ID Number	4621
Wave	26
Anticipated licensing information	CONFIDENTIAL
Draft remit	To appraise the clinical and cost effectiveness of pegaptanib sodium within its licensed indication for the treatment of diabetic macular oedema
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of pegaptanib sodium for the treatment of diabetic macular oedema is appropriate. The proposed remit is appropriate.
Process (MTA/STA)	STA
Proposed changes to remit (in bold)	No changes proposed
Costing implications of remit change	Not applicable no changes proposed
Timeliness statement	Assuming the anticipated date of the marketing authorisation is the latest date that we are aware of and the expected referral date of this topic, issuing timely guidance for this technology will not be possible.

Provisional Title	Plerixafor for haematopoietic stem cell mobilisation prior to autologous stem cell transplantation in the treatment of lymphoma
Topic Selection ID Number	2902
Wave	25
Anticipated licensing information	Marketing authorisation: received in August 2009. Marketing authorisation wording: Plerixafor is indicated in combination with G-CSF to enhance mobilisation of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with lymphoma and multiple myeloma whose cells mobilise poorly.
Draft remit	To appraise the clinical and cost effectiveness of plerixafor within its licensed indication for haematopoietic stem cell mobilisation prior to autologous stem cell transplantation in the treatment of lymphoma.
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of plerixafor for haematopoietic stem cell mobilisation prior to autologous stem cell transplantation in the treatment of lymphoma is <u>not</u> appropriate. The patient population with lymphoma suitable for plerixafor within the terms of its marketing authorisation is estimated to be less than 200 a year. This number is comprised to two distinct patient populations: Hodgkin's lymphoma and non-Hodgkin's lymphoma. The two conditions have different clinical characteristics and need to be considered separately. Plerixafor is currently used in the NHS. It was reported in consultation that 8 out of 10 specialised bone marrow commissioning groups (covering 82% of the treatment population) have procedures in place and patient access is currently believed to be relatively equitable. At the workshop, patient experts suggested that, despite this, variation in availability and practice still exists. However, clinical specialists emphasised that heterogeneity of practice is largely attributable to uncertainty about how, rather than whether, to use plerixafor. For example, the definition of stem cells that mobilise poorly is different in different centres, and there is uncertainty about the optimal place of plerixafor within the treatment pathway. Given the nature of the issues identified at the scoping workshop it is not considered that a technology appraisal of plerixafor for stem cell mobilisation in the treatment of lymphoma would be of value to the NHS.
Process (MTA/STA)	Referral not requested

Proposed changes to remit (in bold)	Referral not requested
Costing implications of remit change	N/A
Timeliness statement	Referral not requested

Provisional Title	Plerixafor for haematopoietic stem cell mobilisation prior to autologous stem cell transplantation in the treatment of multiple myeloma
Topic Selection ID Number	2901
Wave	25
Anticipated licensing information	Marketing authorisation date: received in August 2009. Marketing authorisation wording: Plerixafor is indicated in combination with G-CSF to enhance mobilisation of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with lymphoma and multiple myeloma whose cells mobilise poorly.
Draft remit	To appraise the clinical and cost effectiveness of plerixafor within its licensed indication for haematopoietic stem cell mobilisation prior to autologous stem cell transplantation in the treatment of multiple myeloma.
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of plerixafor for haematopoietic stem cell mobilisation prior to autologous stem cell transplantation in the treatment of multiple myeloma is <u>not</u> appropriate. The patient population with multiple myeloma suitable for plerixafor within the terms of its marketing authorisation is estimated to be less than 100 a year. This patient group is distinct from the lymphoma patient groups and would have to be considered separately in any appraisal. Plerixafor is currently used in the NHS. It was reported in consultation that 8 out of 10 specialised bone marrow commissioning groups (covering 82% of the treatment population) have procedures in place and patient access is currently believed to be relatively equitable. At the workshop, patient experts suggested that, despite this, variation in availability and practice still exists. However, clinical specialists emphasised that heterogeneity of practice is largely attributable to uncertainty about how, rather than whether, to use plerixafor. For example, the definition of stem cells that mobilise poorly is different in different centres, and there is uncertainty about the optimal place of plerixafor within the treatment pathway. Given the nature of the issues identified at the scoping workshop it is not considered that a technology appraisal of plerixafor for stem cell mobilisation in the treatment of multiple myeloma would be of value to the NHS.
Process (MTA/STA)	Referral not requested
Proposed changes to remit (in bold)	Referral not requested

Costing implications of remit change	N/A
Timeliness statement	Referral not requested

Provisional Title	Duloxetine for the treatment of chronic somatic pain
Topic Selection ID Number	4810
Wave	26
Anticipated licensing information	CONFIDENTIAL
Draft remit	To appraise the clinical and cost effectiveness of duloxetine within its licensed indication for the treatment of chronic somatic pain.
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of duloxetine for the treatment of chronic somatic pain is not appropriate. Duloxetine is licensed and in use in the NHS for the treatment of peripheral neuropathic pain, depression, and generalised anxiety disorder. Recommendations for duloxetine for these indications are included in relevant NICE clinical guidelines. Consultees at the workshop stated that these are common co-morbidities with somatic pain. Therefore a person presenting with somatic pain may also have other concurrent conditions for which duloxetine could also be prescribed. There was a lack of consensus as to the care pathway and where duloxetine would be most appropriately positioned. Consultees considered that guidance on the pathway of pain management would be of value rather than consideration of duloxetine in isolation from other aspects of care. An NHS Health trust reported that they considered the use of duloxetine could be handled through the local joint formulary process. Given the indications for which duloxetine is licensed and its current use in these indications, it is not considered that additional NICE technology appraisal guidance specifically for duloxetine for the treatment of chronic somatic pain would provide value to the NHS.
Process (MTA/STA)	Referral not requested
Proposed changes to remit (in bold)	Referral not requested
Costing implications of remit change	N/A
Timeliness statement	Referral not requested

Provisional Title	Percutaneous vertebroplasty and percutaneous kyphoplasty for the treatment of osteoporotic vertebral fractures
Topic Selection ID Number	2784
Wave	22
Anticipated licensing information	Not applicable, devices are already in use in the NHS. Each device has its own CE mark.
Draft remit	To appraise the clinical and cost effectiveness of percutaneous vertebroplasty and balloon kyphoplasty for the treatment of osteoporotic vertebral fractures.
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of vertebroplasty and kyphoplasty for the treatment of osteoporotic vertebral fractures is appropriate. The proposed remit is not appropriate. Clinical specialists explained that balloon kyphoplasty is occasionally performed during open spinal surgery, which would fall beyond the scope of the proposed appraisal. Therefore, the remit should be clarified that balloon kyphoplasty is only of interest in the percutaneous approach. It was explained that vertebral body stents are sometimes introduced to the vertebral cavity following balloon inflation, and that this was considered to be part of the same procedure. Consultees considered that there would be value in NICE appraising balloon kyphoplasty with and without the use of stents and that the use of stents should fall within the scope of the proposed appraisal.
Process (MTA/STA)	MTA
Proposed changes to remit (in bold)	To appraise the clinical and cost effectiveness of percutaneous vertebroplasty and **percutaneous** balloon kyphoplasty **(with or without vertebral body stenting)** for the treatment of osteoporotic vertebral fractures.
Costing implications of remit change	There is no change to the costing implications statement. Guidance on prevention of osteoporotic fragility (TA160 &TA161) published January 2011 has the potential to reduce numbers for whom the intervention is considered. It is still considered to be low cost.
Timeliness statement	Not applicable

Provisional Title	Adalimumab for the treatment of juvenile idiopathic arthritis
Topic Selection ID Number	
Wave	17
Anticipated licensing information	CONFIDENTIAL
Draft remit	To appraise the clinical and cost effectiveness of adalimumab within its licensed indication for the treatment of polyarticular juvenile idiopathic arthritis.
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of adalimumab for the treatment of juvenile idiopathic arthritis is not appropriate. It is estimated that there are 9000 children age 4-17 with juvenile idiopathic arthritis. Of these, approximately 20% will have polyarticular type, and approximately 15% will have disease not adequately controlled by conventional DMARDs. Etanercept is recommended as a treatment for juvenile idiopathic arthritis by NICE technology appraisal number 35 (static list) and would be the main comparator in an appraisal of adalimumab for this indication. Consultees at the scoping workshop indicated that the clinical effectiveness of adalimumab and etanercept is thought to be comparable in juvenile idiopathic arthritis, and both are available at the same price (approximately £360 per two weeks) to the NHS. Using adalimumab rather than etanercept, is in effect a substitution (similar product with similar cost and effect) with no major resource implications additional to those of etanercept. Consultees indicated that adalimumab is currently being used without NICE guidance in children with juvenile idiopathic arthritis (age 13-17 years) and usage is expected to increase should the marketing authorisation be extended. The decision to use either etanercept or adalimumab is often based on parental choice, child preference and factors such as the frequency of the treatment. Given the cost and benefit profile of adalimumab in comparison with etanercept and its existing usage on the NHS, it is not considered that a technology appraisal of adalimumab for juvenile idiopathic arthritis would provide value to the NHS.
Process (MTA/STA)	Referral not requested
Proposed changes to remit (in bold)	Referral not requested
Costing implications of	N/A

remit change	
Timeliness statement	Referral not requested