

**NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE**  
**CENTRE FOR HEALTH TECHNOLOGY EVALUATION**  
**Technology Appraisals**

**Consultation on Batch 46 draft remits and draft scopes and  
summary of comments and discussions at scoping workshops**

| Item | Topic ID | Topic title   |
|------|----------|---|
| 4.2  | 881      | Etirinotecan pegol for treating breast cancer with brain metastases                                     |
| 4.3  | 904      | Ixekizumab for treating moderate to severe chronic plaque psoriasis                                     |
| 4.4  | 866      | Padeliporfin for treating localised prostate cancer   |
| 4.5  | 882      | Abaloparatide for preventing osteoporotic fractures in postmenopausal women                             |
| 4.6  | 921      | Sofosbuvir and velpatasvir for treating chronic hepatitis C   |
| 4.7  | 915      | Palbociclib for treating advanced or metastatic, hormone receptor-positive, HER2-negative breast cancer |
| 4.8  | 917      | MABp1 for previously treated metastatic colorectal cancer   |
| 4.9  | 910      | APN311 for treating high-risk neuroblastoma   |
| 4.10 | 746      | Vosaroxin for treating relapsed or refractory acute myeloid leukaemia                                   |

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| <b>Provisional Title</b>                    | Etirinotecan pegol for treating breast cancer with brain metastases   |                     |      |
| <b>Topic Selection ID Number</b>            | 7264  | <b>Wave / Round</b> | R100 |
| <b>TA ID Number</b>                         | 881   |                     |      |
| <b>Company</b>                              | Nektar Therapeutics   |                     |      |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***  |                     |      |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of etirinotecan pegol within its marketing authorisation for treating locally advanced or metastatic breast cancer previously treated with chemotherapy.  |                     |      |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of etirinotecan pegol for treating metastatic breast cancer with brain metastases is <u>appropriate</u>.</p> <p>The proposed remit is not appropriate and should be amended to specify that the focus of the appraisal is patients with breast cancer and brain metastases. This is to reflect revised marketing authorisation information.</p>  |                     |      |
| <b>Population size</b>                      | <p>Approximately 2000 people in England would be eligible for treatment with etirinotecan pegol.</p> <p>This estimate is based on 44,800 people diagnosed with breast cancer per year, of whom 46% develop metastatic disease (17% at diagnosis, 83%*35% subsequently). Estimates of the number of people with breast cancer and brain metastases range from 5% to 16%, so a mid-point of 10% is assumed. Cancer research UK states that there are no reliable data on the incidence of brain metastases, therefore this estimate is uncertain.</p> |                     |      |
| <b>Process (MTA/STA/HST)</b>                | STA   |                     |      |
| <b>Proposed changes to remit (in bold)</b>  | To appraise the clinical and cost effectiveness of etirinotecan pegol within its marketing authorisation for treating <b>locally advanced or</b> breast cancer <b>with brain metastases previously treated with chemotherapy</b> .  |                     |      |
| <b>Costing implications of remit change</b> | Etirinotecan pegol is an additional option for the treatment of breast cancer with brain metastases. The drug cost and cost impact is currently unknown.  |                     |      |
| <b>Timeliness statement</b>                 | Assuming that 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 be possible.   |                     |      |

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| <b>Provisional Title</b>                    | Ixekizumab for treating moderate to severe chronic plaque psoriasis  |                     |      |
| <b>Topic Selection ID Number</b>            | 7632   | <b>Wave / Round</b> | R129 |
| <b>TA ID Number</b>                         | 904  |                     |      |
| <b>Company</b>                              | Eli Lilly  |                     |      |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***   |                     |      |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of ixekizumab within its marketing authorisation for treating moderate to severe chronic plaque psoriasis.   |                     |      |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of ixekizumab for treating moderate to severe chronic plaque psoriasis is <u>appropriate</u>.</p> <p>The proposed remit is appropriate. No changes are required.</p>  |                     |      |
| <b>Population size</b>                      | <p>Approximately 171,000 people in England would be eligible for treatment with ixekizumab.</p> <p>This estimate is based on the prevalence of psoriasis in England being 1.75%, which is about 951,000 people, of whom about 20% have moderate to severe psoriasis (15% moderate, 5% severe) and 90% have plaque psoriasis.</p> |                     |      |
| <b>Process (MTA/STA/HST)</b>                | STA  |                     |      |
| <b>Proposed changes to remit (in bold)</b>  | To appraise the clinical and cost effectiveness of ixekizumab within its marketing authorisation for treating moderate to severe <del>chronic</del> plaque psoriasis.  |                     |      |
| <b>Costing implications of remit change</b> | The cost of ixekizumab is not yet known. Alternative biological treatment options cost around £9,300–£12,900 per annum; the cost impact will depend on the treatment cost in relation to the alternative options.  |                     |      |
| <b>Timeliness statement</b>                 | Assuming that 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 be possible.  |                     |      |

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| <b>Provisional Title</b>                    | Padeliporfin for treating localised prostate cancer   |                     |      |
| <b>Topic Selection ID Number</b>            | 7714  | <b>Wave / Round</b> | R137 |
| <b>TA ID Number</b>                         | 866   |                     |      |
| <b>Company</b>                              | Steba Biotech   |                     |      |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***  |                     |      |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of padeliporfin within its marketing authorisation for treating localised prostate cancer.  |                     |      |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise, the Institute is of the opinion that an appraisal of padeliporfin for treating localised prostate cancer is <u>appropriate</u>.</p> <p>The proposed remit is appropriate. No changes are required.</p>  |                     |      |
| <b>Population size</b>                      | <p>Up to 19,400 people in England may be eligible for treatment with padeliporfin. <i>Estimate based on 40,400 people diagnosed with prostate cancer per year in England (<a href="#">ONS</a>), of whom 48% have localised (stage I or II) disease at diagnosis (<a href="#">CRUK</a>).</i></p> |                     |      |
| <b>Process (MTA/STA/HST)</b>                | STA   |                     |      |
| <b>Proposed changes to remit (in bold)</b>  | None  |                     |      |
| <b>Costing implications of remit change</b> | The cost of padeliporfin is not yet known. The main comparator is active surveillance, so it seems likely that padeliporfin will increase treatment costs at least in the short-term.   |                     |      |
| <b>Timeliness statement</b>                 | Assuming that 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 be possible.   |                     |      |

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| <b>Provisional Title</b>                    | Abaloparatide for preventing osteoporotic fractures in postmenopausal women  |                     |      |
| <b>Topic Selection ID Number</b>            | 7533   | <b>Wave / Round</b> | R123 |
| <b>TA ID Number</b>                         | 882  |                     |      |
| <b>Company</b>                              | Radius Health  |                     |      |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***   |                     |      |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of abaloparatide within its marketing authorisation for preventing osteoporotic fractures in postmenopausal women.   |                     |      |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of abaloparatide for preventing osteoporotic fractures is <u>appropriate</u>.</p> <p>This was proposed as a single technology appraisal. All attendees at the scoping workshop, except for the company, said they would prefer abaloparatide to be included in the multiple technology appraisal of non-bisphosphonates for osteoporosis (ID901). The MTA is anticipated to start in late 2016 once the ongoing MTA of bisphosphonates has been published. Consultees said that recommendations from an STA would be difficult to interpret in the context of multiple treatment options, and conducting an MTA would be a more efficient use of NICE's resources. In contrast, the company prefers an STA in order to ensure timely guidance.</p> <p>At the decision point 4 meeting, the attendees agreed to include this topic in the proposed MTA of non-bisphosphonates. Although this means the guidance on abaloparatide will be delayed, an MTA will be more informative for clinicians and patients than an STA. However, attendees were mindful that reducing fractures in older people is an area of national priority. Therefore they agreed to explore whether an evidence summary on abaloparatide can be created in the intervening time.</p> <p>The proposed remit is appropriate. No changes are required.</p> |                     |      |
| <b>Population size</b>                      | <p>Up to 53,000 people in England and Wales may be eligible for treatment with abaloparatide.</p> <p><i>Estimate based on:</i></p> <ul style="list-style-type: none"> <li>- About 650,000–750,000 postmenopausal women in England and Wales eligible for treatment.</li> <li>- 93% receive oral bisphosphonates.</li> <li>- Anticipate that abaloparatide will be used for 7% for whom bisphosphonates are not suitable or not tolerated.</li> </ul>   |                     |      |
| <b>Process (MTA/STA/HST)</b>                | Include in MTA of non-bisphosphonates (ID901)  |                     |      |
| <b>Proposed changes to remit (in bold)</b>  | None   |                     |      |
| <b>Costing implications of remit change</b> | <p>The cost of abaloparatide is not known yet. Other treatments exist, with a varied cost range, and so the resource impact will depend on abaloparatide compared to these other treatments.</p> <p>Where falls and fractures are avoided, there could be significant savings from reduced NHS and social care costs.</p>  |                     |      |
| <b>Timeliness statement</b>                 | Assuming that 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   |                     |      |

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|  | guidance for this technology will be possible. |
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| <b>Provisional Title</b>                    | Sofosbuvir and velpatasvir for treating chronic hepatitis C  |                     |      |
| <b>Topic Selection ID Number</b>            | 7630 (combined 7701 and 7700)  | <b>Wave / Round</b> | R133 |
| <b>TA ID Number</b>                         | 921  |                     |      |
| <b>Company</b>                              | Gilead Sciences  |                     |      |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***   |                     |      |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of the combination of sofosbuvir and velpatasvir within its marketing authorisation for treating chronic hepatitis C.  |                     |      |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise, the Institute is of the opinion that an appraisal of sofosbuvir and velpatasvir for treating chronic hepatitis C is <u>appropriate</u>.</p> <p>The proposed remit is appropriate. No changes are required.</p>   |                     |      |
| <b>Population size</b>                      | Approximately 160,000 people in England have been diagnosed with chronic hepatitis C (2012 estimate). It is not known how many people would be eligible for treatment with sofosbuvir–velpatasvir, because the company has not informed NICE of the anticipated place in the treatment pathway, or the target patient groups.  |                     |      |
| <b>Process (MTA/STA/HST)</b>                | STA  |                     |      |
| <b>Proposed changes to remit (in bold)</b>  | None   |                     |      |
| <b>Costing implications of remit change</b> | The cost of the combined treatment is unknown and it would be an alternative treatment option. The cost of a different combination treatment, sofosbuvir–ledipasvir, is £12,993.33 per 28-tablet pack (excluding VAT; based on company submission for TA363). The cost of an 8 week course is 25,986.66, a 12-week course of treatment is £38,979.99 and a 24-week course is £77,959.98 (all excluding VAT). These costs relate to the list price of the treatment and do not reflect contract pricing arrangements between the company and the Commercial Medicines Unit. The contract prices are commercial in confidence. The population size is unknown as the company has not informed NICE of the anticipated eligible patient groups. |                     |      |
| <b>Timeliness statement</b>                 | Assuming that 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 be possible.  |                     |      |

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| <b>Provisional Title</b>                    | Palbociclib for treating advanced or metastatic, hormone receptor-positive, HER2-negative breast cancer   |                     |             |
| <b>Topic Selection ID Number</b>            | 7803 / 7279   | <b>Wave / Round</b> | R144 / R104 |
| <b>TA ID Number</b>                         | 915   |                     |             |
| <b>Company</b>                              | Pfizer  |                     |             |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***  |                     |             |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of palbociclib within its marketing authorisation for treating metastatic hormone receptor-positive, HER2-negative breast cancer.   |                     |             |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of palbociclib for treating hormone receptor-positive, HER2-negative breast cancer is <u>appropriate</u>.</p> <p>The proposed remit is not appropriate and should be amended to include the word 'advanced' to include both metastatic and inoperable locally advanced disease.<br/>***CONFIDENTIAL INFORMATION REMOVED***</p> <p>There are two key clinical trials informing the palbociclib marketing authorisation, one in people with previously untreated advanced or metastatic breast cancer and one in people with advanced or metastatic breast cancer that has been previously treated with endocrine therapy. In one trial palbociclib is combined with letrozole and in the other fulvestrant.</p> <p>The different positions in the treatment pathway mean that the appraisal could be split into two STAs; one for previously untreated advanced or metastatic breast cancer and one for previously treated advanced or metastatic breast cancer. Alternatively a single STA could consider both positions in the pathway. The requested remit covers both the option of a single STA and two STAs without requiring further changes other than that stated above.</p> |                     |             |
| <b>Population size</b>                      | <p>Approximately 8000 people in England would be eligible for treatment with palbociclib.</p> <p>This estimate is based on there being 45,000 new diagnoses of breast cancer each year, of which 75% are for HER2 negative and 70% are ER positive. If 5% of diagnoses present with advanced breast cancer and another 35% of breast cancers recur after treatment for early disease the advanced HER2 negative, ER positive population could be up to 8000.</p>  |                     |             |
| <b>Process (MTA/STA/HST)</b>                | STA   |                     |             |
| <b>Proposed changes to remit (in bold)</b>  | To appraise the clinical and cost effectiveness of palbociclib within its marketing authorisation for treating <b>advanced or</b> metastatic hormone receptor-positive, HER2-negative breast cancer.  |                     |             |
| <b>Costing implications of remit change</b> | The cost of palbociclib is not yet known. It is an additional treatment option for this patient group and so the resource impact will depend on the cost compared with current treatment options.   |                     |             |
| <b>Timeliness statement</b>                 | Assuming that 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 be possible.   |                     |             |



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| <b>Provisional Title</b>                    | MABp1 for previously treated metastatic colorectal cancer  |                     |      |
| <b>Topic Selection ID Number</b>            | 7872   | <b>Wave / Round</b> | R150 |
| <b>TA ID Number</b>                         | 917  |                     |      |
| <b>Company</b>                              | XBiotech   |                     |      |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***   |                     |      |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of MABp1 within its marketing authorisation for treating metastatic or unresectable colorectal cancer after oxaliplatin and irinotecan based regimens.   |                     |      |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise, the Institute is of the opinion that an appraisal of MABp1 for previously treated metastatic colorectal cancer is <u>appropriate</u>.</p> <p>The proposed remit is appropriate. No changes are required.</p>   |                     |      |
| <b>Population size</b>                      | <p>It is unknown how many patients with metastatic colorectal cancer would be eligible for treatment with MABp1 for treating metastatic or unresectable colorectal cancer after oxaliplatin and irinotecan based regimens.</p> <p>Between 10% and 25% of people with colorectal cancer have metastatic disease when first diagnosed, and 50% of people who have surgery for early stage disease will eventually develop metastases. In 2011 around 34,000 people in England were diagnosed with colorectal cancer. Therefore in 2011 there were approximately between 20,400 and 25,500 of patients with metastatic colorectal cancer.</p> |                     |      |
| <b>Process (MTA/STA/HST)</b>                | STA  |                     |      |
| <b>Proposed changes to remit (in bold)</b>  | None   |                     |      |
| <b>Costing implications of remit change</b> | The cost of MABp1 is not yet known. As it is an alternative treatment option the cost impact depends on the cost of MABp1 compared to the alternative options. There may be additional administration costs compared to current treatment options since MABp1 is administered by IV infusion.  |                     |      |
| <b>Timeliness statement</b>                 | Assuming that 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 be possible.  |                     |      |

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| <b>Provisional Title</b>                    | APN311 for treating high-risk neuroblastoma  |                     |      |
| <b>Topic Selection ID Number</b>            | 7862   | <b>Wave / Round</b> | R149 |
| <b>TA ID Number</b>                         | 910  |                     |      |
| <b>Company</b>                              | Apeiron  |                     |      |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***   |                     |      |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of APN311 within its marketing authorisation for treating high-risk neuroblastoma following myeloablative therapy and autologous stem cell transplant.                                 |                     |      |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise, the Institute is of the opinion that an appraisal of APN311 for treating high-risk neuroblastoma is <u>appropriate</u>.</p> <p>The proposed remit is appropriate. No changes are required.</p> |                     |      |
| <b>Population size</b>                      | It is estimated that 70 children in England are diagnosed with neuroblastoma each year, around 40 of whom are high risk and would be eligible for treatment with APN311.   |                     |      |
| <b>Process (MTA/STA/HST)</b>                | STA  |                     |      |
| <b>Proposed changes to remit (in bold)</b>  | None   |                     |      |
| <b>Costing implications of remit change</b> | The cost of APN311 is not yet known. Any costs would be additional for the NHS as this treatment is currently received as part of clinical trials and there are very few effective therapies currently available on the NHS.           |                     |      |
| <b>Timeliness statement</b>                 | Assuming that 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 be possible.                  |                     |      |

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| <b>Provisional Title</b>                    | Vosaroxin for treating relapsed or refractory acute myeloid leukaemia  |                     |     |
| <b>Topic Selection ID Number</b>            | 6248   | <b>Wave / Round</b> | R32 |
| <b>TA ID Number</b>                         | 746  |                     |     |
| <b>Company</b>                              | Sunesis  |                     |     |
| <b>Anticipated licensing information</b>    | ***CONFIDENTIAL INFORMATION REMOVED***   |                     |     |
| <b>Draft remit</b>                          | To appraise the clinical and cost effectiveness of vosaroxin within its marketing authorisation for treating relapsed or refractory acute myeloid leukaemia.   |                     |     |
| <b>Main points from consultation</b>        | <p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of vosaroxin for treating relapsed or refractory acute myeloid leukaemia is <u>appropriate</u>.</p> <p>The proposed remit is appropriate. No changes are required.</p>  |                     |     |
| <b>Population size</b>                      | <p>The incidence of AML in England is about 2,500 per year. Around three quarters of all cases occur in those over 60 years (Cancer research UK) that will be around 1,900 people per year.</p> <p>A proportion of those 1,900 people will receive induction chemotherapy (excluding those who die before induction or ineligible for induction chemotherapy) and ultimately be diagnosed with relapsed or refractory disease (<a href="#">Ramos et al. 2015</a>).</p> |                     |     |
| <b>Process (MTA/STA/HST)</b>                | STA  |                     |     |
| <b>Proposed changes to remit (in bold)</b>  | None   |                     |     |
| <b>Costing implications of remit change</b> | The cost of vosaroxin is not yet known. As it is an alternative treatment option the cost impact depends on the cost of vosaroxin compared to the alternative options.   |                     |     |
| <b>Timeliness statement</b>                 | Assuming that 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 be possible.  |                     |     |