NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Technology Appraisals

Consultation on Batch 61 draft remits and draft scopes and Summary of comments and discussions at scoping workshops

Topic ID	Topic title
ID1105	Avatrombopag for treating thrombocytopenia in people with chronic liver disease needing an elective procedure
ID1398	Risankizumab for treating chronic moderate to severe plaque psoriasis
ID1397	Rivaroxaban for preventing major cardiovascular events in people with coronary or peripheral artery disease
ID1110	Pegvaliase for treating phenylketonuria
ID1035	Erythropoiesis stimulating agents for treating anaemia in people with myelodysplastic syndromes
ID1101	Andexanet alfa for reversing anticoagulation

Provisional Title	Avatrombopag for treating thrombocytopenia in people with chronic liver disease needing an elective procedure		
Topic Selection ID Number	7508	**Wave / Round**	R118
TA ID Number	1105		
Company	Dova Pharmaceuticals		
Anticipated licensing information	***confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of avatrombopag within its marketing authorisation for treating thrombocytopenia in people with chronic liver disease needing elective surgery.		
Main points from consultation	Following the consultation exercise, NICE is of the opinion that an appraisal of avatrombopag for treating thrombocytopenia in adults with chronic liver disease needed an elective procedure is <u>appropriate.</u> The proposed remit is not appropriate and should be amended as follows: 'To appraise the clinical and cost effectiveness of avatrombopag within its marketing authorisation for treating thrombocytopenia in people with chronic liver disease needing an elective procedure.' The word 'surgery' has been changed to 'procedure' to encompass both surgery and more minor procedures for which the treatment may be used. ***Confidential information removed*** At DP3, the decision makers suggested that this could be appraised as an MTA with ID1149 lusutrombopag for the same indication ***Confidential information removed***. A question about the appropriateness of an MTA was asked in the consultation for this scope (ID1105). The company stated that *'Dova recommends that Avatrombopag is assessed by NICE under an STA and not under an MTA given the likely EC approval and UK launch timing for Avatrombopag'*. No further comments on the appropriateness of an MTA were received. Considering the anticipated dates of marketing authorisation for both drugs, some efficiencies could be gained by appraising together within an MTA. Therefore, it is recommended this is appraised as an MTA.		
Population size	Approximately 3,071 people in England would be eligible for treatment with avatrombopag This is based on lusutrombopag (Shionogi) market research which suggests that 11% of the 27,927 hospital admissions (HES data) for people with liver disease in England could receive lusutrombopag.		
Process (TA/HST)	TA - MTA (with ID1149 lusutrombopag – both individual remits will be used)		

Proposed changes to remit (in bold)	To appraise the clinical and cost effectiveness of avatrombopag within its marketing authorisation for treating thrombocytopenia in people with chronic liver disease needing **an** elective **procedure ~~surgery~~**.
Costing implications	The cost of avatrombopag is unknown and therefore the resource impact is unknown. There may be savings as a result of a reduced need for platelet transfusions.
Timeliness statement	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 MTA guidance for this technology will be possible.

Provisional Title	Risankizumab for treating chronic moderate to severe plaque psoriasis		
Topic Selection ID Number	9191	**Wave / Round**	R239
TA ID Number	1398		
Company	AbbVie		
Anticipated licensing information	***confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of risankizumab within its marketing authorisation for treating moderate to severe plaque psoriasis		
Main points from consultation	Following the consultation exercise, NICE is of the opinion that an appraisal of risankizumab for treating moderate to severe plaque psoriasis is appropriate. The proposed remit is appropriate. No changes are required. The company suggested that a cost comparison approach is most appropriate for this technology, with comparison to the most recently appraised IL-23 inhibitor treatment guselkumab, given guselkumab represents the relevant class of drugs with similar mechanism of action to risankizumab. One other stakeholder agreed with this approach, others either made no comment or disagreed without giving a rationale. Potential for cost comparison FTA		
Population size	Approximately 2,595 to 4,391 people in England would be eligible for treatment with risankizumab. This estimate is based on 508,748 to 860,959 people being diagnosed in England each year with plaque psoriasis, of whom 20% have moderate or severe disease, of whom 2.55% are eligible for this treatment. (**Source:** Costing comment)		
Process (TA/HST)	TA		
Proposed changes to remit (in bold)	None		
Costing implications	If licensed, risankizumab will offer an additional treatment option. The cost of risankizumab is not yet known however, because it is an additional treatment option alongside many other existing biological therapies any potential resource impact is unlikely to be significant. For information, the cost of a similar treatment, guselkumab (without PAS) is around £17,700 for the first year and maintenance treatment is around £14,600 per person.		
Timeliness statement	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.		

Provisional Title	Rivaroxaban for preventing major cardiovascular events in people with coronary or peripheral artery disease.		
Topic Selection ID Number	9140	**Wave / Round**	R236
TA ID Number	1397		
Company	Bayer		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of rivaroxaban within its marketing authorisation for preventing major cardiovascular events in people with coronary or peripheral artery disease.		
Main points from consultation	Following the consultation exercise and the scoping workshop, NICE is of the opinion that an appraisal of rivaroxaban for preventing major cardiovascular events in people with coronary or peripheral artery disease is <u>appropriate.</u> The Committee for Medicinal Products for Human Use adopted a positive opinion recommending a change to the terms of the marketing authorisation for the medicinal product Xarelto (rivaroxaban) Based on this change the proposed remit is not appropriate and should be amended as follows: "To appraise the clinical and cost effectiveness of rivaroxaban within its marketing authorisation for preventing <u>atherothrombotic</u> ~~major cardiovascular~~ events in people with coronary or peripheral artery disease <u>at high risk of ischaemic events</u>."		
Population size	The company anticipate that approximately 437,100 people in England would be eligible for treatment with rivaroxaban.		
Process (TA/HST)	TA		
Proposed changes to remit (in bold)	None		
Costing implications	Rivaroxaban is given orally as 2.5mg twice daily in combination with aspirin given orally as 100mg once daily. The annual treatment cost per person for treatment with rivaroxaban is around £660 there will also be a small annual charge for aspirin of around £15 (BNF). If uptake is 1% of the 437,100 eligible people costs for rivaroxaban will be around £2,872,000, As rivaroxaban will be a new treatment option for these people there may be offsetting costs depending on what treatments people are currently receiving.		
Timeliness statement	Considering that this product is shortly due to receive a marketing authorisation for use in the UK, publication of timely guidance will not be possible. NICE were not notified of the amendment to the marketing authorisation date early enough to be able to provide the opportunity to issue timely guidance.		

Provisional Title	Pegvaliase for treating phenylketonuria		
Topic Selection ID Number	8432	**Wave / Round**	R195
TA ID Number	1110		
Company	BioMarin		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of pegvaliase within its marketing authorisation for treating phenylketonuria		
Main points from consultation	Following the consultation exercise and the scoping workshop, NICE is of the opinion that an appraisal of pegvaliase for treating phenylketonuria is appropriate. The proposed remit is appropriate. No changes are required. The comparator in the scope is established clinical management without pegvaliase. This consists of a Phe restricted diet. Although sapropterin is licensed for treating phenylketonuria it is only commissioned by NHS England for pregnant women. Sapropterin has also been formally referred to NICE [ID1475]. Stakeholders suggested that nutritional outcomes are important, especially during the transition phase to a normal diet; nutritional biochemistry (for example, vitamin B12) has been added to the scope. The company stated that the OPTIC study, a phase 3b study recruiting its first patient in October 2018, will be critical in informing an appraisal of pegvaliase. The study will explore the impact of more flexible dietary requirements, change in dosing schedule to manage adverse events, and include quality of life tools. The company strongly support delaying the start of an appraisal to align with the results of the OPTIC study ***Confidential information removed***		
Population size	Approximately 3,400 people in England have PKU. It is not known how many of these would have Phe concentration in blood>600 µmol/L and then be eligible for treatment with pegvaliase. This estimate comes from the NICE Resource Impact Assessment team (May 2016)		
Process (TA/HST)	TA		
Proposed changes to remit (in bold)	None		
Costing implications	The cost of pegvaliase is not yet known and therefore the resource impact is not known, however, because pegvaliase will be available for first line use and as an adjunct to the PKU diet, there will be additional costs.		

Timeliness statement	NICE's opportunity to publish timely guidance will depend on the date of availability of the OPTIC study, if the appraisal is delayed to allow the inclusion of these results.

Provisional Title	Epoetin alfa for treating anaemia in people with myelodysplastic syndromes		
Topic Selection ID Number	8420	**Wave / Round**	R193
TA ID Number	1035		
Company	Janssen		
Anticipated licensing information	Marketing authorisation for epoetin alfa (Eprex) granted April 2017 for treatment of symptomatic anaemia (haemoglobin concentration of ≤10 g/dL) in adults with low- or intermediate-1-risk primary myelodysplastic syndromes (MDS) who have low serum erythropoietin (<200 mU/mL).		
Draft remit	To appraise the clinical and cost effectiveness of epoetin alfa within its marketing authorisation for treating anaemia in people with myelodysplastic syndromes.		
Main points from consultation	Following the consultation exercise and the scoping workshop, NICE is of the opinion that an appraisal of erythropoiesis stimulating agents for treating anaemia is <u>not appropriate.</u> There were mixed views about the appropriateness of an appraisal. Some consultees felt that the use of erythropoiesis stimulating agents (ESAs) is embedded in clinical practice for this indication, therefore an appraisal would not be necessary. Other stakeholders stated that there was variation in access to ESAs across England, and considered that an MTA which would include all relevant ESAs and not just epoetin alfa alone would be valuable. NICE previously appraised all the ESAs together in an MTA (TA323) for anaemia in people with cancer having chemotherapy. NICE recognises that ESAs are used in clinical practice in England already for this indication and other types of anaemia albeit with some slight variation in access. However, NICE does not consider that a multiple technology appraisal of the ESAs would add value to the NHS.		
Population size	Approximately 2000 people in England would be eligible for treatment with an ESA each year. This takes into account: • The incidence of MDS - Annual incidence of MDS approximately 4 per 100,000 (increases to 30/100,000 per year in people over 70). In 2013 there were 2,335 people newly diagnosed with MDS in England. • Most people will be anaemic at some stage (40% at diagnosis and 80% during the course of their disease).		
Process (TA/HST)	Not applicable – referral not sought		
Proposed changes to remit (in bold)	Not applicable – referral not sought		

Costing implications	Using on an average adult weight of 78kg the cost of epoetin alfa is expected to range between £9,300 and £28,400 per person per year. This is based on the manufacturer's estimates of treatment with 337.5 – 1,050 IU/kg once weekly. However, the company states that the technology is subject to the NHS national tendering process and the eMit price will apply in this case.
Timeliness statement	Considering that this product has received a marketing authorisation for use in the UK, publication of timely guidance will not be possible.

Provisional Title	Andexanet alfa for reversing anticoagulation		
Topic Selection ID Number	8347	**Wave / Round**	R188
TA ID Number	1101		
Manufacturer	Portola Pharmaceuticals		
Anticipated licensing information	***confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of andexanet alfa within its marketing authorisation for reversing anticoagulation		
Main points from consultation	Following the consultation exercise and the scoping workshop, and following further information and reconsideration, the Institute is of the opinion that an appraisal of andexanet alfa for reversing anticoagulation is appropriate. The proposed remit is appropriate. No changes are required. NICE recognises that this is a potentially valuable new treatment option; bearing in mind the anticipated size of the population, cost implications for the NHS and potential variation in practice, a technology appraisal is appropriate. Following the consultation, the scope has been updated to more precisely define the population, comparators and outcomes for the appraisal.		
Population size	Approximately 3150–6300 people in England would be eligible for treatment with andexanet alfa. *Source: company estimate. Scoping workshop attendees estimated that 350,000 people were receiving DOACs in 2015, of whom 1–2% experience significant or life-threatening bleeding requiring reversal of anticoagulation, which is consistent with this figure.*		
Process (TA/HST)	TA		
Proposed changes to remit (in bold)	None		
Costing implications of remit change	The unit cost of andexanet alfa is unknown so the resource impact of this technology cannot currently be estimated.		