

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

**Consultation on Batch 60 draft remits and draft scopes and
summary of comments and discussions at scoping workshops**

Topic ID	Topic title
ID968	LentiGlobin for treating transfusion-dependent beta-thalassaemia
ID1268	Lanadelumab for the long-term prevention of angioedema attacks in hereditary angioedema types I and II
ID1364	Pentosan polysulfate sodium for treating bladder pain syndrome
ID1368	Fremanezumab for preventing migraine
ID1362	Canakinumab for preventing cardiovascular events after myocardial infarction in people with raised high-sensitivity C-reactive protein
ID1178	Avacopan for inducing remission in anti-neutrophil cytoplasmic antibody-associated vasculitis
ID1475	Sapropterin for treating phenylketonuria
ID1320	Rituximab for maintenance treatment of anti-neutrophil cytoplasmic antibody-associated vasculitis
ID1378	Fingolimod - oral for multiple sclerosis; relapsing remitting, in paediatric patients aged 10-17 years first line.
ID1032	Drug-eluting beads loaded with irinotecan for treating liver metastases in colorectal cancer
ID999	Drug-eluting beads loaded with doxorubicin for treating hepatocellular carcinoma

Provisional Title	LentiGlobin for treating transfusion-dependent beta-thalassaemia		
Topic Selection ID Number	8111	Wave / Round	R169
TA ID Number	968		
Company	Bluebird Bio		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of LentiGlobin within its marketing authorisation for treating beta-thalassaemia major		
Main points from consultation	<p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of LentiGlobin for treating transfusion-dependent beta-thalassaemia is <u>appropriate</u>.</p> <p>The draft remit is not appropriate and should be amended as follows: the term 'transfusion-dependent beta-thalassaemia' should be used instead of 'beta-thalassaemia major', as this is the preferred clinical term and reflects the wording of the anticipated MA.</p> <p>Stakeholders agreed that LentiGlobin would be a potentially curative gene therapy for people eligible for haematopoietic stem cell transplantation (HSCT) who do not have access to a matched related donor and who therefore require regular blood transfusions and chelating agents to manage the disease.</p> <p>The company believes that the appraisal is more suited to the highly specialised technologies (HST) process. ***Confidential information removed***</p> <p>The Institute is of the opinion that beta thalassaemia with ***Confidential information removed*** are not clinically distinct populations. The symptoms and possible treatment options are the same for these different populations. Furthermore the mechanism of action of LentiGlobin is expected to be the same in all of these populations. Clinicians at the scoping workshop confirmed that genetic testing in beta-thalassaemia is part of routine clinical practice in the UK and that beta-thalassaemia ***Confidential information removed*** is genetically distinct, but the results are not used to guide the choice between different treatment options.</p> <p>Clinical experts stated that the life expectancy of people with transfusion-dependent beta-thalassaemia has improved dramatically with people now living into their sixth and seventh decade of life, and that quality of life with transfusions and chelation therapy can be good.</p> <p>In terms of population size, the company estimates that half of beta-thalassaemia patients (approximately 270), have ***Confidential information removed***. As myeloablative chemotherapy is given before LentiGlobin, with associated risks of short-term toxicity and infertility over the longer-term, the</p>		

	<p>company expects only a subset of these to be fit for, and to want to, undergo treatment. There were a range of views among other stakeholders on what proportion of the 270 patients with ***Confidential information removed*** would want to undergo treatment. This would depend on age of patient and fertility considerations, cultural and religious factors, and satisfaction and quality of life with existing treatment, which the clinical experts reported can be good (see above).</p> <p>Currently, there are a number of specialist centres that look after patients undergoing transfusions and chelation therapy. However, there is an infrastructural and organisational change ongoing and the aim is to centralise the care into 3-4 specialist hub centres. Stakeholders at the scoping workshop said that this change might be in place by the end of 2018. It is unlikely that LentiGlobin would be used outside of these centres, however it is not expected that the technology will be used and commissioned exclusively within the context of a highly specialised service.</p>
Population size	<p>Up to 270 people in England would be eligible for treatment with LentiGlobin. This is based on:</p> <ul style="list-style-type: none"> • Hospital Episode Statistics, which suggest there are 537 people with transfusion-dependent beta-thalassaemia in England aged 12-50 years (the age of patients in the trial). • Approximately half of beta-thalassaemia patients have ***Confidential information removed***, equating to 270 patients (based on information from the Oxford Reference Molecular Haematology Laboratory and the Royal London Hospital, provided by the company). • However, it is anticipated that only a subset of the 270 patients would be fit for, and want to, undergo treatment.
Process (TA/HST)	TA
Proposed changes to remit (in bold)	To appraise the clinical and cost effectiveness of LentiGlobin within its marketing authorisation for treating transfusion-dependent beta-thalassaemia major
Costing implications	The unit cost of LentiGlobin is unknown so the resource impact of this technology cannot currently be estimated.
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	Lanadelumab for the long-term prevention of angioedema attacks in hereditary angioedema types I and II		
Topic Selection ID Number	8968	Wave / Round	R227
TA ID Number	1268		
Company	Shire Pharmaceuticals		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of lanadelumab within its marketing authorisation for the long-term prevention of angioedema attacks in hereditary angioedema types I and II		
Main points from consultation	<p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of lanadelumab for the long-term prevention of angioedema attacks in hereditary angioedema types I and II is <u>appropriate</u>.</p> <p>The draft remit is appropriate. No changes are required.</p> <p>Attendees at the workshop discussed the expected position of lanadelumab within the treatment pathway for hereditary angioedema (HAE). The population in the scope has been amended to include a definition of 'frequent attacks' which is consistent with the expected use of lanadelumab in the treatment pathway.</p>		
Population size	<p>Approximately 50-1,000 people in England would be eligible for treatment with lanadelumab.</p> <p>The number of people eligible for treatment with lanadelumab is unknown as it is dependent on the definition of 'frequent' angioedema attacks.</p> <p>It is estimated that HAE affects between 1 per 10,000 to 1 per 50,000 of the population.</p> <p>The clinical trial (HELP) only includes patients that have at least 1 attack per 4 weeks. The number of people who have at least 1 attack per 4 weeks is unknown, but HSRIC notes that about 500-1,000 patients with HAE and that on average they suffer between 3-6 attacks per year.</p> <p>NHS England's clinical commissioning policy on the use of C1-esterase inhibitors (comparator in the scope) for prophylactic treatment of HAE types I and II estimates that 50-100 people (adults and children) may experience 2 or more clinically significant attacks per week who may benefit from long-term prophylactic C1-esterase inhibitors (see NHS England [2016] Clinical Commissioning Policy: Plasma derived C1-esterase inhibitor for prophylactic treatment of hereditary angioedema (HAE) types I and II).</p>		
Process (TA/HST)	TA		

Proposed changes to remit (in bold)	None
Costing implications	The unit cost of lanadelumab is unknown so the resource impact of this technology cannot currently be estimated.
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	Pentosan polysulfate sodium for treating bladder pain syndrome		
Topic Selection ID Number	9517	Wave / Round	R255
TA ID Number	1364		
Company	Consilient Health		
Licensing information	Marketing authorisation granted: June 2017 UK launch anticipated: ***Confidential information removed*** Wording of marketing authorisation: “for the treatment of bladder pain syndrome with glomerulations or Hunner’s lesions, in adults with moderate to severe pain, urgency and frequency of micturition”		
Draft remit	To appraise the clinical and cost effectiveness of pentosan polysulfate sodium within its marketing authorisation for treating interstitial cystitis.		
Main points from consultation	<p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of pentosan polysulfate sodium for treating bladder pain syndrome is <u>appropriate</u>.</p> <p>The draft remit is not appropriate and should be amended as follows: The term ‘bladder pain syndrome’ is used in place of ‘interstitial cystitis’ – stakeholders highlighted that this term is more appropriate and is consistent with the marketing authorisation.</p> <p>Stakeholders highlighted that the need for a technology appraisal is uncertain. The population eligible for treatment within the marketing authorisation is small, the technology is already used in the NHS (on a named-patient basis), and no new evidence has become available since publication of ESUOM43. However, stakeholders suggested that patients are keen to access the technology, and there is currently variation in availability across the country.</p> <p>Stakeholders indicated that the technology would be considered as an alternative to bladder instillations or, if these were unsuccessful or unsuitable, established clinical management. The comparators have been updated accordingly.</p>		
Population size	<p>Approximately 9000 people in England would be eligible for treatment with pentosan polysulfate sodium.</p> <p><i>Source: company estimate, based on an estimated prevalence for bladder pain syndrome characterised by glomerulations or Hunner’s lesions of up to 1.8 per 10,000. The company noted that actual use may be lower given the limited use of cystoscopy with hydrodistension, which is required to confirm glomerulations and Hunner’s lesions.</i></p>		
Process (TA/HST)	TA		
Proposed changes to remit (in bold)	To appraise the clinical and cost effectiveness of pentosan polysulfate sodium within its marketing authorisation for treating interstitial cystitis bladder pain syndrome .		
Costing implications	The unit cost of pentosan polysulfate sodium is unknown so the resource impact of this technology cannot currently be estimated.		

Timeliness statement	Assuming that the anticipated commercial launch date is the latest date that we are aware of and the expected referral date of this topic, issuing timely guidance from date of launch of this technology will be possible.
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Provisional Title	Fremanezumab for preventing migraine		
Topic Selection ID Number	9571 (combined with 9754)	Wave / Round	R263 / R281
TA ID Number	1368		
Company	Teva Pharmaceuticals		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of fremanezumab within its marketing authorisation for preventing migraine.		
Main points from consultation	<p>Following the consultation exercise, the Institute is of the opinion that an appraisal of fremanezumab for preventing migraine is <u>appropriate</u>.</p> <p>The draft remit is appropriate. No changes are required.</p> <p>Currently proposed as an STA.</p>		
Population size	The prevalence of chronic migraine is estimated to be approximately 15% of the population in England. Limited data on the prevalence or incidence of episodic migraine.		
Process (TA/HST)	TA		
Proposed changes to remit (in bold)	None		
Costing implications	The unit cost of fremanezumab is unknown so the resource impact of this technology cannot currently be estimated.		
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	Canakinumab for preventing cardiovascular events after myocardial infarction in people with raised high-sensitivity C-reactive protein		
Topic Selection ID Number	8430	Wave / Round	R194
TA ID Number	1362		
Company	Novartis		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of canakinumab within its marketing authorisation for preventing cardiovascular events after myocardial infarction in people with raised high-sensitivity C-reactive protein.		
Main points from consultation	<p>Following the consultation exercise, the Institute is of the opinion that an appraisal of canakinumab for preventing cardiovascular events after myocardial infarction in people with raised high-sensitivity C-reactive protein treating is <u>appropriate</u>.</p> <p>The draft remit is appropriate. No changes are required.</p> <p>Canakinumab is expected to be an add-on to standard therapy for preventing cardiovascular events in people who have had an acute myocardial infarction and are at high risk of further events. The intervention in the scope has been amended to be consistent with canakinumab's expected use.</p>		
Population size	<p>Approximately 48,000 people in England could be eligible for treatment with canakinumab.</p> <p>81,000 hospital admissions for acute myocardial infarction in England in 2016/17. Research sponsored by company suggests that 58.6% of people on 2 US registries had an elevated hsCRP 30 days after a myocardial infarction.</p>		
Process (TA/HST)	TA		
Proposed changes to remit (in bold)	None		
Costing implications	<p>The annual treatment cost is around £39,700 per person at list price (assuming quarterly administration of a 150mg dose). Canakinumab is an add-on therapy and so will be an additional cost to the NHS. Based on this, if uptake is around 1% of the 48,000 people potentially eligible for treatment, the additional annual drug costs would be around £19 million.</p> <p>There may be offsetting savings from a reduction in costs associated with treating other cardiovascular events which cannot currently be quantified.</p>		
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	Avacopan for inducing remission in anti-neutrophil cytoplasmic antibody-associated vasculitis		
Topic Selection ID Number	8781	Wave / Round	R212
TA ID Number	1178		
Company	Vifor Fresenius Medical Care Renal Pharma		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of avacopan within its marketing authorisation for treating anti-neutrophil cytoplasmic antibody-associated vasculitis		
Main points from consultation	<p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of avacopan for treating ANCA-associated vasculitis <u>appropriate</u>.</p> <p>The draft remit is not appropriate and should be amended as follows: 'induction of remission' has been added, consistent with the CLEAR trial ***Confidential information removed***</p> <p>Stakeholders indicated that avacopan will be used in combination with rituximab or cyclophosphamide; the intervention and comparators have been amended accordingly.</p> <p>It is uncertain whether avacopan will be used in combination with corticosteroids (at a full or reduced dose), or will replace them fully within the treatment regimen. The scope has been left broad to allow discussion of this during the appraisal.</p>		
Population size	<p>Approximately 1500–5000 people in England would be eligible for treatment with avacopan.</p> <p><i>Source: TA308 – company estimated that 5000 people per year have induction therapy for vasculitis, of whom one-third have severe disease (consistent with MA for rituximab).</i></p> <p><i>Estimated prevalence of ANCA-associated vasculitis in England is 11,400 (200 per million [Watts et al. 2012] applied to ONS mid-year population estimate for 2015)</i></p>		
Process (TA/HST)	TA		
Proposed changes to remit (in bold)	To appraise the clinical and cost effectiveness of avacopan within its marketing authorisation for treating inducing remission in anti-neutrophil cytoplasmic antibody-associated vasculitis		
Costing implications	The unit cost of avacopan is unknown so the resource impact of this technology cannot currently be estimated.		
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	Sapropterin for treating phenylketonuria		
Topic Selection ID Number	9859	Round	305
TA ID Number	1475		
Company	BioMarin		
Licensing information	<p>Marketing authorisation granted: December 2008</p> <p>Wording of marketing authorisation: Kuvan is indicated for the treatment of hyperphenylalaninaemia (HPA) in adults and paediatric patients of all ages with phenylketonuria (PKU) who have been shown to be responsive to such treatment</p>		
Draft remit	To appraise the clinical and cost effectiveness of sapropterin within its marketing authorisation for treating phenylketonuria		
Process (TA/HST)	TA		

Provisional Title	Rituximab for maintenance treatment of anti-neutrophil cytoplasmic antibody-associated vasculitis		
Topic Selection ID Number	9284	Wave / Round	R283
TA ID Number	1320		
Company	Roche		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of rituximab within its marketing authorisation for maintenance treatment of anti-neutrophil cytoplasmic antibody-associated vasculitis		
Main points from consultation	<p>Following the consultation exercise, the Institute is of the opinion that an appraisal of rituximab for treating anti-neutrophil cytoplasmic antibody-associated vasculitis is <u>not appropriate</u>.</p> <p>Rationale:</p> <ul style="list-style-type: none"> • Rituximab for maintenance is used in clinical practice, NHSE commission maintenance therapy with rituximab (off-label) for a subgroup of patients. • Recent European clinical practice guidelines recommend treatment with a combination of low-dose glucocorticoids and either azathioprine, rituximab, methotrexate or mycophenolate mofetil for remission maintenance. • The patent for rituximab has expired and biosimilars (Truxima and Rixathon) are now available in the UK. <p>Note: The company state that they will not be submitting for this topic.</p>		
Population size	<p>The expected population size in England that would be eligible for treatment with rituximab is unknown.</p> <ul style="list-style-type: none"> • The total number of people living with vasculitis (GPA and MPA) in England is 11,400 <p>(Watts et al. 2012, applied to ONS mid-year population estimate for 2015)</p> <p>NHS England has estimated that approximately “40 to 70 people in each senate region will need rituximab (including maintenance treatment) each year” This may be equivalent to <1000 people in England (there are 12 senate regions in England)</p>		
Process (TA/HST)	Not applicable – referral not sought		
Proposed changes to remit (in bold)	Not applicable – referral not sought		
Costing implications	Since the expected population in England that would be eligible for treatment with rituximab is unknown, the resource impact of this technology cannot currently be estimated.		
Timeliness statement	Not applicable – referral not sought		

Provisional Title	Fingolimod for treating relapsing multiple sclerosis in children and young people		
Topic Selection ID Number	8398	Wave / Round	R192
TA ID Number	1378		
Company	Novartis		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To appraise the clinical and cost effectiveness of fingolimod within its marketing authorisation for treating relapsing multiple sclerosis in children and young people.		
Main points from consultation	<p>Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of fingolimod for treating relapsing multiple sclerosis in children and young people is <u>not appropriate</u>.</p> <p>Stakeholders (including the company and NHS England) suggest that fingolimod for children and young people is already available through the NHS England's 'Commissioning Medicines for Children in specialised Services' policy for 2nd line treatment (that is after disease modifying therapies).</p> <p>However, if the CHMP opinion ***Confidential information removed*** covers patient groups that are not covered in published guidance or policies then the institute will reconsider its opinion.</p> <p>The remit for fingolimod (adults; TA254) is <i>"To appraise the clinical and cost effectiveness of fingolimod within its licensed indication for the treatment of relapsing-remitting multiple sclerosis"</i>. The original TA254 remit is sufficiently broad to include the indication for children and young people.</p> <p>Issues relating to comparators and outcomes have been addressed directly in the scope. Specifically adding additional treatments used outside of their marketing authorisation as comparators, and outcomes relating to carers.</p>		
Population size	Approximately 160 people aged between 10 and 19 years in the UK have multiple sclerosis, and about 60 people are diagnosed each year (source: Multiple Sclerosis Society).		
Process (TA/HST)	N/A – referral not sought		
Proposed changes to remit	N/A – referral not sought		
Costing implications	Fingolimod will likely be administered orally daily at a dose of either 0.5 mg or 0.25 mg depending on the person's body weight. Fingolimod is available with a patient access scheme with a confidential price of ***Confidential information removed***. In adults, the annual treatment cost per person is around ***Confidential information removed*** per person, if administered using the 0.5 mg dose. However, as the eligible population is small this is unlikely to be a high cost drug.		
Timeliness statement	N/A – referral not sought		

Provisional Title	Drug-eluting beads loaded with irinotecan for treating liver metastases in colorectal cancer		
Topic Selection ID Number	8316	Wave / Round	R184
TA ID Number	1032		
Company	BTG, Terumo, Merit, Boston Scientific		
CE Mark information	<p>All technologies have a CE mark.</p> <p>Wording of CE marking:</p> <p>DC Bead & DC Bead M1 (BTG):</p> <ul style="list-style-type: none"> Intended to embolise the vessels supplying malignant colorectal cancer metastasised to the liver As a secondary action, will elute a local, controlled and sustained dose [of irinotecan] to the liver metastases from colorectal cancer after embolisation <p>DC Bead LUMI (BTG):</p> <ul style="list-style-type: none"> [Same indication as DC Bead & DC Bead M1] <p>LifePearl (Terumo):</p> <ul style="list-style-type: none"> Indicated for embolization of blood vessels supplying primary-hypervascular tumours or metastases in the liver Note: microspheres can be loaded with chemotherapeutic drugs (such as doxorubicin, epirubicin, idarubicin, irinotecan) <p>HepaSphere (Merit):</p> <ul style="list-style-type: none"> Loaded with irinotecan: indicated for use in embolization of metastatic colorectal cancer to the liver [and] embolization of metastases to the liver <p>Embozene Tandem (Boston Scientific):</p> <ul style="list-style-type: none"> Indicated for embolization of hypervascular tumours, arteriovenous malformations [and] hepatocellular carcinoma Note: can be loaded with doxorubicin or irinotecan <p>[Indicated for other embolic procedures unrelated to this scope]</p>		
Draft remit	To appraise the clinical and cost effectiveness of drug-eluting beads loaded with irinotecan within its approved indication for treating liver metastases in colorectal cancer.		

Main points from consultation	<p>Following the consultation exercise and the scoping workshop and discussions with the MHRA, the Institute is of the opinion that an appraisal of drug-eluting beads for treating colorectal cancer metastases predominantly in the liver is <u>not appropriate</u>.</p> <p>Although consultees and commentators were supportive of an appraisal of this technology, the regulatory status of the drug component of the technology precludes a full technology appraisal with funding direction. This route of administration and posology is outside the marketing authorisation for irinotecan. Neither the medical device regulations nor the medicines regulations legislate for this type of drug-device combination.</p> <p>Because the drug component is not authorised for use in this indication, a technology appraisal cannot be formally referred within regulation 7. Instead a referral would need to be sought via regulation 5 and not carry any formal funding requirements. A technology appraisal without the support of a funding requirement is unlikely to add value in this area. Given the complexities around the regulatory status of these technologies, it may be appropriate for these technologies to be considered within other NICE products (such as a medtech innovation briefing combined with / or other types of NICE advice).</p>
Population size	<p>Fewer than 25,000 to 27,000 people in England would be eligible for treatment with drug-eluting beads.</p> <p>This estimate is based on 34,952 people diagnosed with colorectal cancer in England in 2016, of whom 23 to 26% have metastatic disease at diagnosis and 50% of people who have surgery for early stage disease eventually develop metastases.</p>
Process (TA/HST)	N/A – referral not sought
Proposed changes to remit (in bold)	N/A – referral not sought
Costing implications	Unknown. Costs for the various technologies vary from around £650 to £1350, but it is unclear how many doses would be used in practice. Any resource impact will also depend on the incremental cost compared to the comparator, of which there are several. The overall resource impact is therefore unknown.
Timeliness statement	N/A – referral not sought

Provisional Title	Drug-eluting beads loaded with doxorubicin for treating hepatocellular carcinoma		
Topic Selection ID Number	8128	Wave / Round	R172
TA ID Number	999		
Company	BTG, Merit, Terumo and Boston Scientific		
CE Mark information	<p>All technologies have a CE mark.</p> <p>Wording of CE marking:</p> <p>DC Bead & DC Bead M1 (BTG):</p> <ul style="list-style-type: none"> Primarily intended as an embolic agent for the local treatment of malignant hypervascularised tumour(s) in the liver As a secondary action, will elute a local, controlled and sustained dose [of doxorubicin] to the tumour after embolization <p>DC Bead LUMI (BTG):</p> <ul style="list-style-type: none"> [Same indication as DC Bead & DC Bead M1] [In addition,] unloaded DC Bead LUMI is intended to be used for the embolization of non-malignant hypervascular tumours and arteriovenous malformations <p>LifePearl (Terumo):</p> <ul style="list-style-type: none"> Indicated for embolization of blood vessels supplying primary-hypervascular tumours or metastases in the liver Note: microspheres can be loaded with chemotherapeutic drugs <p>HepaSphere (Merit):</p> <ul style="list-style-type: none"> Indicated for use in embolization of blood vessels with or without delivery of doxorubicin HCl for therapeutic or preoperative purposes in the embolization of hepatocellular carcinoma [and] the embolization of metastases to the liver <p>Embozene Tandem (Boston Scientific):</p> <ul style="list-style-type: none"> Indicated for embolization of hypervascular tumours, arteriovenous malformations [and] hepatocellular carcinoma <p>[Indicated for other embolic procedures unrelated to this scope]</p>		
Draft remit	To appraise the clinical and cost effectiveness of drug-eluting beads loaded with doxorubicin within their approved indications for treating hepatocellular carcinoma		

Main points from consultation	<p>Following the consultation exercise, the scoping workshop and discussions with regulatory bodies, NICE is of the opinion that an appraisal of drug-eluting beads for treating hepatocellular carcinoma is <u>not appropriate</u>.</p> <p>There is consensus that drug-eluting beads loaded with doxorubicin are already routinely used in the NHS for HCC. However clinical experts and companies considered an appraisal was appropriate because it would make it easier to access drug-eluting bead transarterial chemoembolisation if it was on the tariff. Stakeholders noted that although the procedure was funded, the technologies were not. Although consultees and commentators were supportive of an appraisal of this technology, the regulatory status of the drug component of the technology precludes a full technology appraisal with funding direction. This route of administration and posology is outside the marketing authorisation for doxorubicin. Neither the medical device regulations nor the medicines regulations legislate for this type of drug-device combination.</p> <p>Because the use of the drug is not authorised for use, a technology appraisal cannot be formally referred within regulation 7. Instead a referral would need to be sought via regulation 5 and not carry any formal funding requirements. A technology appraisal without the support of a funding requirement is unlikely to add value in this area. Given the complexities around the regulatory status of these technologies, it may be appropriate for these technologies to be considered within other NICE products (such as a medtech innovation briefing combined with / or other types of NICE advice)..</p>
Population size	<p>Approximately 838 people in England would be eligible for treatment with drug-eluting beads loaded with doxorubicin (estimate for new cases per-year; based on incidence).</p> <p>Number of people with liver cancer diagnoses = 4925</p> <p>Proportion with HCC = $4925 \times 0.85 = 4186$</p> <p>Proportion with intermediate HCC = $4186 \times 0.2 = 838$</p>
Process (TA/HST)	N/A – referral not sought
Proposed changes to remit (in bold)	N/A – referral not sought
Costing implications	Costs for the various technologies vary from around £650 to £1350. However, since the technologies are already routinely used to treat HCC in the NHS, additional costs are not expected as a result of this appraisal.
Timeliness statement	N/A – referral not sought