NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Proposed Highly Specialised Technology Evaluation

Afamelanotide for treating erythropoietic protoporphyria [ID927]

Consultee and commentator comment form

Please use this form for submitting your comments on the draft remit, draft scope and provisional matrix of consultees and commentators. It is important that you complete and return this form even if you have no comments otherwise we may chase you for a response.

Enter the name of your organisation here: British Association of Dermatologists

Comments on the draft remit and draft scope

The draft remit is the brief for a proposed evaluation. Appendix B contains the draft remit. The draft scope, developed from the draft remit outlines the question that the proposed evaluation would answer.

Please submit your comments on the draft remit and draft scope using the table below. **Please take note of any questions that have been highlighted in the draft scope itself** (usually found at the end of the document).

If you have been asked to comment on documents for more than one proposed evaluation, please use a separate comment form for each topic, even if the issues are similar.

Please complete this form and upload it to NICE Docs by Thursday 3 March 2016. If using NICE docs is not possible please return via email to scopingta@nice.org.uk If you have any questions please contact Michelle Adhemar, Project Manager on 44 (0)20 7045 2239 or at the email address above.

If you do not have any comments to make on the draft remit and draft scope, please state this in the box below.

Comment 1: the draft remit

Section	*Notes*	Your comments
Appropriateness	*It is important that appropriate topics are referred to NICE to ensure that NICE guidance is relevant, timely and addresses priority issues, which will help improve the health of the population. Would it be appropriate to refer this topic to NICE for evaluation?*	Yes. Totally appropriate. Afamelanotide is potentially an important therapeutic development for patients with erythropoietic protoporphyria.
Wording	*Does the wording of the remit reflect the issue(s) of clinical and cost effectiveness about this technology or technologies that NICE should consider? If not,*	Yes.

Section	*Notes*	Your comments
	please suggest alternative wording.	
Timing Issues	*What is the relative urgency of this proposed evaluation to national commissioning by NHS England?*	URGENT; progress needs to be made as rapidly as possible to alleviate the considerable suffering of EPP patients.
Any additional comments on the draft remit		

Comment 2: the draft scope

Section	*Notes*	Your comments
Background information	*Consider the accuracy and completeness of this information.*	Inaccurate - understates severity of condition: 'when the face or hands of a person with EPP are exposed to sun, the porphyrin mediated damage causes necrosis of skin small blood vessel endothelium resulting invariably in 2-3 days of severe and intense burning pain during which the patient experiences burning pain usually described as 'like having burning oil poured on the skin'. The pain is unresponsive to all analgesia apart from opioids.'
The technology/ intervention	*Is the description of the technology or technologies accurate?*	Yes.
Population	*Is the population defined appropriately? Are there groups within this population that should be considered separately?*	Yes it is defined accurately. No subgroups to consider separately.
Comparators	*Is this (are these) the standard treatment(s) currently used with which the technology should be compared? Can this (one of these) be described as 'best alternative care'?*	Yes that is correct - i.e. there is no effective treatment currently.
Outcomes	*Will these outcome measures capture the most important health related benefits (and harms) of the technology?*	Meaningful outcome measures are a significant issue here. There is a consensus in the porphyria academic and clincial community that the outcomes measured in the trials (which are the ones listed here) have underestimated the therapeutic effects significantly (when compared to qualitative experience of patients).
Equality	*NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if*	It seems unfair to exclude teenage patients for whom there is no biological or scientific reason to assume that the drug is any less effective or more dangerous than in adults. Teenage

Section	*Notes*	Your comments
	you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope: • *could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which [the treatment(s)] is/are/will be licensed;* • *could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;* • *could have any adverse impact on people with a particular disability or disabilities.* *Please tell us what evidence should be obtained to enable the Highly Specialised Technologies Evaluation Committee to identify and consider such impacts.*	patients have a paticularly high impact on quality of life from this disease.
Other considerations	*Suggestions for additional issues to be covered by the proposed evaluation are welcome.*	None.
Innovation	*Do you consider the technology to be innovative in its potential to make a significant and substantial impact on health-related benefits and how it might improve the way that current need is met (is this a 'step-change' in the management of the condition)?*	Yes it is a step change - it is the first effective treatment in EPP.
Questions for consultation	*Please answer any of the questions for consultation if not covered in the above sections. If appropriate, please include comments on the proposed process this evaluation will follow (please note any changes made to the process are likely to result in changes to the planned time lines).*	1) There are no relevant comparators - the treatments we currently have are not effective. Best supportive care should include 'visible light photoprotection'. 2) There are seven Photodermatology tertiary Departments in the UK, all of which see EPP patients. 85 patients followed up in Cutaneous Porphyrias Clinic at Guy's Hospital. National highly Specialist centrally commissioned Acute Porphyria Service does not treat EPP. Diagnostic labs for EPP are the same porphyrin labs as for Acute Porphyria National Service.
Any additional comments on the draft scope		

Comment 3: provisional matrix of consultees and commentators

The provisional matrix of consultees and commentators (Appendix C) is a list of organisations that we have identified as being appropriate to participate in this proposed evaluation. If you have any comments on this list, please submit them in the box below.

As NICE is committed to promoting equality and eliminating unlawful discrimination, please let us know if we have missed any important organisations from the lists contained within the matrix, and which organisations we should include that have a particular focus on relevant equality issues.

If you do not have any comments to make on the provisional matrix of consultees and commentators, please cross this box: ☒

Comments on the provisional matrix of consultees and commentators

Comment 4: regulatory issues

Section	*Notes*	Your comments
Remit	*Does the wording of the remit reflect the current or proposed marketing authorisation? If not, please suggest alternative wording.*	
Current or proposed marketing authorisation	*What are the current indications for the technology?*	
	What are the planned indications for the technology?	
	FOR EACH PLANNED INDICATION:	
	What is the target date (mm/yyyy) for regulatory submission?	
	Which regulatory process are you following?	
	What is the anticipated date (mm/yyyy) of CHMP positive opinion (if applicable) and regulatory approval?	
	Please indicate whether the information you provide concerning the proposed marketing authorisation is in the public domain and if not when it can be released. All commercial in confidence information must be highlighted and underlined.	

Section	*Notes*	Your comments
Economic model software	*NICE accepts executable economic models using standard software, that is, Excel , DATA, R or WinBUGs. Please indicate which software will be used. If you plan to submit a model in a non-standard package, NICE, in association with the ERG, will investigate whether the requested software is acceptable, and establish if you need to provide NICE and the ERG with temporary licences for the non –standard software for the duration of the evaluation. NICE reserves the right to reject economic models in non-standard software*	

Please complete this form and upload it to NICE Docs by Thursday 3 March 2016. If using NICE docs is not possible please return via email to scopingta@nice.org.uk If you have any questions please contact Michelle Adhemar, Project Manager on 44 (0)20 7045 2239 or at above the email address.