NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 52 draft remits and draft scopes and summary of comments and discussions at scoping workshops

Topic ID	Topic title
861	Metreleptin for treating lipodystrophy
927 (B47)	Afamelanotide for erythropoietic protoporphyria

Provisional Title	Metreleptin for treating lipodystrophy		
Topic Selection ID Number	7412	**Wave / Round**	112
TA/HST ID Number	861		
Company	Aegerion Pharmaceuticals		
Anticipated licensing information	***CONFIDENTIAL INFORMATION REMOVED***		
Draft remit	To appraise the clinical and cost effectiveness of metreleptin within its marketing authorisation for treating lipodystrophy.		
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an evaluation of metreleptin for treating lipodystrophy is <u>appropriate</u>. The proposed remit is not appropriate. It should be amended to match the standard wording for HST evaluations, no other changes are required. Consultees considered that this topic would be suitable for an HST evaluation, rather than an STA. Based on the information provided during the consultation, including refined population size estimates, the NICE technical team proposes that the HST prioritisation criteria are met and an HST evaluation would be appropriate. Other minor issues (including the descriptions of the disease, comparators, outcomes and subgroups) have been addressed directly in the scope.		
Population size	Approximately 100 people in England are expected to be eligible for treatment with metreleptin. *Source: consultees provided evidence from a number of sources which consistently suggested the prevalence of lipodystrophy is approx. 4 per million (including 2.5–3 per mil with partial LD and <1 per mil with generalised LD), suggesting there are approx. 200 people with LD in England.* ***CONFIDENTIAL INFORMATION REMOVED***		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	To evaluate the ~~**clinical and cost effectiveness**~~ **benefits and costs** of metreleptin within its ~~**marketing authorisation**~~ **licensed indication** for treating lipodystrophy **for national commissioning by NHS England**.		
Costing implications of remit change	The price for metreleptin is not yet known. However, this is a new treatment option for this patient group for whom there are currently no specifically licensed treatments available. Therefore any drug costs incurred will be additional for the NHS. There may be some off-setting savings from reduced symptoms and disability.		
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	Afamelanotide for erythropoietic protoporphyria		
Topic Selection ID Number	7867	**Round**	R149
TA ID Number	927		
Company	Clinuvel		
Anticipated licensing information	Marketing authorisation date: December 2014 Marketing authorisation: Afamelanotide has a UK marketing authorisation under exceptional circumstances for 'prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP)'		
Draft remit	To appraise the clinical and cost effectiveness of afamelanotide within its marketing authorisation for treating erythropoietic protoporphyria.		
Main points from consultation	This topic was originally scoped as an HST topic but during consultation a comment was received that the patient population was over 1000 patients in England and the topic was subsequently referred as an STA. Following formal referral of this topic as an STA, the company, clinicians and patient organisations stated that the patient numbers in the scope were now incorrect and provided updated estimates. It was also clarified that there are 4 main treatment centres. Based on the updated estimates, and the fact that all other topic selection criteria for HST are considered to have been met; the decision making group agreed that a formal referral for this topic as a HST is requested. The remit should be as follows: 'To evaluate the benefits and costs of afamelanotide within its licensed indication for treating erythropoietic protoporphyria for national commissioning by NHS England.' The current referral for a STA will be void.		
Population size	400-500 patients with EPP in the UK, and a proportion of these patients would be eligible for treatment with afamelotide.		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	To **evaluate** ~~**appraise**~~ the **benefits and costs** ~~**clinical and cost effectiveness**~~ of afamelanotide within its **licensed indication** ~~**marketing authorisation**~~ for treating erythropoietic protoporphyria **for national commissioning by NHS England**.		
Costing implications of remit change	The proposed remit change does not affect the population size. Afamelanotide is administered as a subcutaneous implant every 60 days. The drug price is not available on the PPA drug tariff, eMit or BNF. There are currently no pharmacological treatments licensed for this condition. There may be some savings from the avoidance of treatment costs for liver failure, which occurs in 1-4% of people with EPP.		
Timeliness	As this technology has already received a marketing		

statement	authorisation, issuing timely guidance for this technology will not be possible. NICE were only notified of the development of the product post the CHMP opinion, which is not early enough in its development in order to provide the opportunity to issue timely guidance.