NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 48 draft remits and draft scopes and summary of comments and discussions at scoping workshops

Topic ID	Topic title
780	Alipogene tiparvovec for treating familial lipoprotein lipase deficiency

Provisional Title	Alipogene tiparvovec for treating familial lipoprotein lipase deficiency		
Topic Selection ID Number	3822	**Wave / Round**	R167
HST ID Number	780		
Company	Chiesi and UniQure		
Anticipated licensing information	Marketing authorisation granted November 2012 Wording of marketing authorisation: 'for adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein.'		
Draft remit	To evaluate the benefits and costs of alipogene tiparvovec within its licensed indication for treating adults with familial lipoprotein lipase deficiency for national commissioning by NHS England.		
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an evaluation of alipogene tiparvovec for treating adults with familial lipoprotein lipase deficiency is <u>not appropriate: :</u> • Only 1 person in the world has received this technology outside a clinical trial since the MA was granted in 2012, • The number of patients who would meet the strict eligibility criteria is exceptionally small.		
Population size	Up to approximately 10-15 people in England would be eligible for treatment with alipogene tiparvovec (but see comment above on number of people actually treated). *Source: company estimate reported during the scoping workshop (estimated 13 people across the UK). Prevalence of LPLD is 1–2 per million, but only a fraction will be eligible for treatment within the marketing authorisation (that is, genetically confirmed LPLD with detectable LPL protein).*		
Process (HTA/HST)	Highly Specialised Technologies		
Proposed changes to remit (in bold)	None		
Costing implications of remit change	Approximately 10-15 people in England would be eligible for treatment with alipogene tiparvovec. The treatment cost of alipogene tiparvovec is not yet known and therefore the resource impact cannot be estimated. Alipogene tiparvovec is the first drug treatment for this condition and so is likely to be an additional cost to the NHS.		
Timeliness statement	Considering that Alipogene tiparvovec has already received a marketing authorisation for this indication, publication of timely guidance will not be possible.		