NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 36 draft remit and draft scope and summary of comments and discussions at the scoping workshop

Provisional Title	Elosulfase alfa for treating mucopolysaccharidosis type IVA		
Topic Selection ID Number	5983	**Wave / Round**	n/a
HST ID Number	744		
Manufacturer	BioMarin Pharmaceuticals		
Anticipated licensing information	UK marketing authorisation was granted in April 2014. Marketing authorisation: Elosulfase alfa (Vimizim) is indicated for the treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome, MPS IVA) in patients of all ages.		
Draft remit	To evaluate the benefits and costs of elosulfase alfa within its licensed indication for the treatment of mucopolysaccharidosis type IVA 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 a highly specialised technology evaluation of elosulfase alfa for treating mucopolysaccharidosis type IVA is appropriate. The proposed remit is appropriate. No changes are required. Stakeholders confirmed that there are no other active treatments for mucopolysaccharidosis type IVA, so best supportive care is the appropriate comparator. They considered that the term 'best supportive care' is open to interpretation, and that the comparator may be more clearly defined as 'established clinical management without elosulfase alfa'. The comparator in the scope has been amended accordingly. Endurance, fatigue and pain were considered to be the 3 most important outcomes to patients, and therefore stakeholders suggested that they should be added to the scope.		
Population size	94 patients in England (all recorded in a national patient registry which is believed to contain all patients)		
Process	HST		
Proposed changes to remit	None		
Costing implications of remit change	N/A – no changes to the remit. No previous costing work has been provided for this topic by costing team. ***CONFIDENTIAL INFORMATION REMOVED***		
Timeliness statement	Given the expected referral date of this topic and the knowledge that this technology received a marketing authorisation in April 2014, issuing timely guidance for this technology will not be possible.		