NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 51 draft remits and draft scopes and summary of comments and discussions at scoping workshops

Topic ID	Topic title
800	Velmanase alfa for treating alpha-mannosidosis

Provisional Title	Velmanase alfa for treating alpha-mannosidosis		
Topic Selection ID Number	7413	**Wave / Round**	R112
HST ID Number	800		
Company	Chiesi		
Anticipated licensing information	***Confidential information removed***		
Draft remit	To evaluate the benefits and costs of recombinant human alpha-mannosidase within its licensed indication for treating alpha-mannosidosis 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 velmanase alfa for treating alpha-mannosidosis is <u>appropriate.</u> The proposed remit is not appropriate and should be amended as follows to include the confirmed name of the technology: To evaluate the benefits and costs of velmanase alfa within its licensed indication for treating alpha-mannosidosis for national commissioning by NHS England. There was one main change to the scope: stakeholders suggested exploring subgroups based on age as younger populations are expected to benefit most from treatment. This has been added to the 'other considerations' section. The company confirmed that the expected population would be aged 6 and over in line with the clinical trials Treatment with velmanase alfa would be considered for all diagnosed with alpha-mannosidase regardless of symptoms or severity as the disease is progressive and the biological processes underpinning the disease are the same regardless of the clinical manifestations.		
Population size	Approximately 12 people in England would currently be eligible for treatment with velmanase alfa, based on information from the Mucopolysaccharidosis Society (MPS).		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	To evaluate the benefits and costs of ~~**recombinant human alpha-mannosidase**~~ **velmanase alfa** within its licensed indication for treating alpha-mannosidosis for national commissioning by NHS England.		
Costing implications of remit change	There is currently only one disease-modifying treatment option available for the condition; however this is not suitable for most people. Therefore the cost of treatment, which is not yet known, would represent an additional cost to the NHS. However if the burden of the disease was reduced, there may be savings to the NHS from reduced hospitalisations and drugs prescribed to manage the condition. The treatment is not currently licensed for use in the UK.		
Timeliness	Assuming that the anticipated date of the marketing		

statement	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.