NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 40 draft remit and draft scope and summary of comments and discussions at scoping workshops

Batch 40
Ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene

Provisional Title	**Ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene**		
Topic Selection ID Number	4383	**Wave / Round**	24
HST ID Number	428		
Company	PTC Therapeutics		
Anticipated licensing information	Ataluren has a conditional marketing authorisation granted in July 2014. Ataluren is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older.		
Draft remit	To evaluate the benefits and costs of ataluren within its marketing authorisation for treating Duchenne muscular dystrophy, resulting from a nonsense mutation in the dystrophin gene for national commissioning by NHS England.		
Main points from consultation	<u>Process</u> • Patient and clinical experts emphasised the urgency of access to ataluren given that many patients who are ambulatory could lose ambulation in the time it takes to reach a decision and would therefore no longer be able to receive ataluren. • On the other hand, the company highlighted that the conditional marketing authorisation requires the provision of further data from an ongoing confirmatory trial. These results will not be available until autumn 2015 at the earliest and the company was concerned that the phase IIb clinical trial on which the conditional marketing authorisation was granted did not meet the primary endpoints and would not be satisfactory for an evaluation. The company suggested delaying the timelines until these data become available. The NICE team explained that it will be possible to submit further data during the evaluation process. The company stated this would be acceptable. • Patient experts suggested that interim funding by NHS England was a matter of urgency. NHS England explained that this would be unlikely to be in place before late summer 2015 at the earliest given ongoing consultation on its decision-making process. • Patient experts stated that many treatments are expected to become available in the near future for treating DMD and asked for a fast moving process to ensure guidance is available as soon as possible for populations with high unmet need who require urgent access to treatment. <u>Scope</u> • Consultees agreed that the remit in the draft scope is appropriate.		

	• Consultees agreed that time to wheelchair and numbers of falls were important outcome measures to be added to the scope. They noted that wheelchair dependency has a devastating impact on health-related quality of life, financial impact on families and can also have an impact on life expectancy by weakening pulmonary and cardiac functions. • Consultees confirmed that girls who have a clinical diagnosis of DMD with a nonsense mutation in the dystrophin gene, follow the natural history of the disease and its management is exactly the same as for boys.
Population size	Around 10 boys are born with the condition each year in the UK.
Process (MTA/STA/HST)	HST
Proposed changes to remit (in bold)	No changes proposed.
Costing implications of remit change	The usual dosage is 40mg/kg daily. The average cost per patient per year is approximately £220,000 based on a median age of 7-9 years and weight of 24kg-26kg.
Timeliness statement	As the technology received a marketing authorisation in July 2014, issuing timely guidance will not be possible.