NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 50 draft remits and draft scopes and summary of comments and discussions at scoping workshops

Topic ID	Topic title
1003	Eteplirsen for treating Duchenne muscular dystrophy
943	Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2

Provisional Title	Eteplirsen for treating Duchenne muscular dystrophy		
Topic Selection ID Number	8109	**Wave / Round**	R169
HST ID Number	1003		
Company	Sarepta Therapeutics		
Anticipated licensing information	*** Commercial in confidence text removed***		
Draft remit	To evaluate the benefits and costs of eteplirsen within its licensed indication for treating of Duchenne muscular dystrophy 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 eteplirsen for treating Duchenne muscular dystrophy is <u>appropriate.</u> The proposed remit is appropriate. A minor typographical error has been corrected, but no other changes are required. Consultees considered an evaluation appropriate, and few substantive changes to the scope were suggested (for example scoping workshop attendees requested clarification of some outcomes). The company anticipates that the marketing authorisation will include both ambulant and non-ambulant patients, although evidence for the latter may be limited; the remit covers the full population.		
Population size	Approximately 138–325 people in England would be eligible for treatment with eteplirsen. *Approximately 1,260–2,500 people in England have Duchenne muscular dystrophy, of whom 11–13% have a mutation that is amenable to eteplirsen therapy.*		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	To evaluate the benefits and costs of eteplirsen within its licensed indication for treating ~~**of**~~ Duchenne muscular dystrophy for national commissioning by NHS England.		
Costing implications of remit change	Eteplirsen would provide a novel, potentially disease-modifying treatment option for this patient group. The cost of eteplirsen is not yet known but, because it is a novel treatment option, would increase drug costs for the NHS. Eteplirsen is administered in hospital weekly and therefore there would be administration costs and an impact on NHS resources.		
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	Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2		
Topic Selection ID Number	8093	**Wave / Round**	R167
HST ID Number	943		
Company	BioMarin		
Anticipated licensing information	*** Commercial in confidence text removed***		
Draft remit	To evaluate the benefits and costs of cerliponase alfa within its licensed indication for treating neuronal ceroid lipofuscinosis type 2 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 cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 (CLN2) is appropriate. The proposed remit is appropriate. No changes are required. Consultees considered that an evaluation is appropriate and urgent, given the unmet need of people with CLN2. They suggested a number of changes to clarify the scope (in particular, the background and outcomes sections), but these did not affect the remit or population under consideration. Consultees noted that there is currently no specific service for CLN2 commissioned in the NHS, but that the availability of cerliponase alfa may encourage development of such a service. If appropriate, the evaluation will consider the costs and implications of any changes in service delivery for CLN2 that may accompany the introduction of the technology.		
Population size	Approximately 30–50 people in England would be eligible for treatment with cerliponase alfa. *Source: estimates provided during consultation by Evelina London Children's Hospital and Genetic Alliance UK. The Batten Disease Family Association estimates that 5–6 people are diagnosed with CLN2 per year in England.*		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	None		
Costing implications of remit change	There are currently no disease modifying or curative treatment options for people with neuronal ceroid lipofuscinosis type 2. The cost of cerliponase alfa is not known but would represent an additional cost for the NHS. Cerliponase alfa is administered in hospital every 2 weeks and therefore there would be administration costs and an impact on NHS resources.		
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.		