NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 37b draft remit and draft scope and summary of comments and discussions at scoping workshops

	Batch 37b
5.1	Asfotase alfa for treating paediatric-onset hypophosphatasia

Provisional Title	Asfotase alfa for treating paediatric-onset hypophosphatasia		
Topic Selection ID Number	7202	**Wave / Round**	R92
HST ID Number	**758**		
Manufacturer	Alexion Pharma UK		
Anticipated licensing information	**CONFIDENTIAL INFORMATION REMOVED**		
Draft remit	To evaluate the benefits and costs of asfotase alfa within its licensed indication for treating paediatric-onset hypophosphatasia 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 asfotase alfa for treating paediatric-onset hypophosphatasia is appropriate. The remit is appropriate. Craniosynotosis and resulting intracranial pressure and pain should be added as additional outcomes in the scope. The population should remain as people with paediatric-onset hypophosphatasia • adult-onset hypophosphatasia were not expected to be included in the marketing authorisation • however, some people may receive a diagnosis during adulthood but they may have had symptoms that could be retrospectively attributed to an onset of hypophosphatasia in their childhood • as a consequence of the difficulties in diagnosing hypophosphatasia and the time it can take for a diagnosis to be made the time of onset of symptoms may be difficult to establish • manufacturer stated that there was unlikely to be difficulty in separating adults with paediatric-onset hypophosphatasia from those with adult-onset hypophosphatasia because paediatric-onset would be associated with the accumulation of fractures and disability over time • population size for this evaluation will be dependent on whether retrospective determination of age of onset is permitted within the marketing authorisation for asfotase alfa The scoping workshop attendees agreed that it would be appropriate to assess the benefits of asfotase alfa for people with different symptom severity separately (infantile-onset and childhood-onset) if evidence allows but highlighted that • there are difficulties in defining discrete patient populations and the symptoms experienced by people with hypophosphatasia vary		
Population size	Available data are based on European estimates and are grouped by severity. Based on an incidence rate of 1/300,000 live births for the most severe forms, there would be expected to be around 2-3 children affected each year in England		

	(assuming a birth rate of ~700,000). Milder forms of hypophosphatasia are estimated to be present in 1 per 6,370 of the population. However not all of the people with milder forms would have a paediatric-onset hypophosphatasia.
Process (MTA/STA/HST)	HST
Proposed changes to remit (in bold)	No changes proposed
Costing implications of remit change	Updated to reflect that we do not use the 'high cost' threshold for HSTs: The cost impact of this technology is unknown but will depend on the severity of disease for which it is recommended. The unit cost of the drug is unknown and it is also not yet known if the drug will be licensed as a first-line treatment for people with severe, or moderate hypophosphatasia. There are currently no effective comparator therapies available and therefore no potential offsetting savings.
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.