NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 55 draft remits and draft scopes and summary of comments and discussions at scoping workshops

Topic ID	Topic title
1151	Burosumab for treating X-linked hypophosphataemia

Provisional Title	Burosumab for treating X-linked hypophosphataemia		
Topic Selection ID Number	8455	**Wave / Round**	R198
HST ID Number	1151		
Company	Kyowa Kirin		
Anticipated licensing information	***CONFIDENTIAL INFORMATION REMOVED***		
Draft remit	To evaluate the benefits and costs of KRN23 within its licensed indication for treating hypophosphataemia for national commissioning by NHS England		
Main points from consultation	Following the consultation exercise and the scoping workshop, NICE is of the opinion that an evaluation of burosumab for treating X-linked hypophosphataemia is appropriate. The remit should be amended to include the updated name of the technology and specify X-linked hypophosphataemia in line with the evidence base and likely marketing authorisation. The team recognises the potential for a future indication in adults, and that this would not be suitable for HST because of population size. However an STA for the combined population may not be suitable because: • this would require delaying the appraisal ***CONFIDENTIAL INFORMATION REMOVED*** proceeding at risk if the adult population does not receive a licence. ***CONFIDENTIAL INFORMATION REMOVED*** • guidance in children was considered valuable because the technology potentially enables children to reach adulthood with better built skeletons. A subsequent adult population could be appraised via STA, and the review date for HST guidance could be aligned with this license extension.		
Population size	Approximately 250 children in England would be eligible for treatment with burosumab. • *This is based on company estimates provided during the consultation and in the workshop.*		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	To evaluate the benefits and costs of ~~**KRN23**~~**burosumab** within its licensed indication for treating **X-linked** hypophosphataemia for national commissioning by NHS England.		
Costing implications	If licensed, KRN23 would represent a new first line treatment. Annual costs are estimated to be in excess of £30,000 per patient. This cost may be offset by a reduction in surgical procedures for musculoskeletal symptoms carried out on this population in later years.		
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.