NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 56 draft remits and draft scopes and summary of comments and discussions at scoping workshops

Topic ID	Topic title
1054	Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations

Provisional Title	Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations		
Topic Selection ID Number	7880	**Wave / Round**	R152
ID Number	1054		
Manufacturer	Spark Therapeutics		
Anticipated licensing information	*** Confidential information removed ***		
Draft remit	To appraise the clinical and cost effectiveness of voretigene neparvovec within its marketing authorisation for treating inherited retinal dystrophies caused by RPE65 gene mutations.		
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of voretigene neparvovec for treating vision loss due to Leber congenital amaurosis or retinitis pigmentosa inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations is appropriate. The proposed remit is appropriate. No changes are required. The topic selection panel consider that all the criteria are met and this topic is suitable for HST The company's comments referred to legislation in support of the HST route.		
Population size	Approximately 100 to 200 people in England would be eligible for treatment with voretigene neparvovec. This is based on clinical expert input at the scoping workshop and consultation comments from the company.		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	None, other than to change to HST wording: To **evaluate the benefits and costs** of voretigene neparvovec within its licensed indication for treating inherited retinal dystrophies caused by RPE65 gene mutations **for national commissioning by NHS England.**		
Costing comments	If voretigene neparvovec is licensed it will be the first sight improving treatment option for this patient group. The price and therefore the potential resource impact of this technology is not yet known.		
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.		