NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 58 draft remits and draft scopes and Summary of comments and discussions at scoping workshops

Topic ID	Topic title
1242	Inotersen for treating hereditary transthyretin-related amyloidosis
1279	Patisiran for treating hereditary transthyretin-related amyloidosis
1326	Volanesorsen for treating familial chylomicronaemia syndrome

Provisional Title	Inotersen for treating hereditary transthyretin-related amyloidosis		
Topic Selection ID Number	8399	**Wave / Round**	192
HST ID Number	1242		
Company	Ionis Pharmaceuticals		
Anticipated licensing information	***CONFIDENTIAL INFORMATION REMOVED***		
Draft remit	To evaluate the benefits and costs of inotersen within its marketing authorisation for treating hereditary transthyretin-related amyloidosis 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 inotersen for treating hereditary transthyretin-related amyloidosis (hATTR) is appropriate. The proposed remit is appropriate. No changes are required. Stakeholders highlighted that people with hATTR amyloidosis in England most commonly have both polyneuropathy and cardiomyopathy symptoms, and that treatment with inotersen would not be restricted to one or the other; therefore, the population in the scope has been broadened to specify 'people with hereditary transthyretin-related amyloidosis'. ***CONFIDENTIAL INFORMATION REMOVED*** Stakeholders agreed that evaluation through HST would be appropriate. They confirmed that the expected population size was approximately 150 patients. Management of hATTR amyloidosis is organised through a Highly Specialised Service at a single centre (National Amyloidosis Centre at UCL). Stakeholders also commented on the outcomes specified in the scope, and these were amended accordingly.		
Population size	Approximately 150 people in England would be eligible for treatment with inotersen. *Estimated by the National Amyloidosis Centre.*		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	None		
Costing implications	Unknown; the cost of inotersen is not yet known so the overall resource impact cannot currently be estimated. For those people who would use inotersen, there would be some offsetting savings across both the NHS and social care.		
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	Patisiran for treating hereditary transthyretin-related amyloidosis		
Topic Selection ID Number	8445	**Wave / Round**	197
HST ID Number	1279		
Company	Alynlam Pharmaceuticals		
Anticipated licensing information	***CONFIDENTIAL INFORMATION REMOVED***		
Draft remit	To evaluate the benefits and costs of patisiran within its marketing authorisation for treating hereditary transthyretin-related amyloidosis 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 patisiran for treating hereditary transthyretin-related amyloidosis (hATTR) is appropriate. The proposed remit is appropriate. No changes are required. Stakeholders highlighted that people with hATTR amyloidosis in England most commonly have both polyneuropathy and cardiomyopathy symptoms, and that treatment with inotersen would not be restricted to one or the other; therefore, the population in the scope has been broadened to specify 'people with hereditary transthyretin-related amyloidosis'. ***CONFIDENTIAL INFORMATION REMOVED*** Stakeholders agreed that evaluation through HST would be appropriate. They confirmed that the expected population size was approximately 150 patients. Management of hATTR amyloidosis is organised through a Highly Specialised Service at a single centre (National Amyloidosis Centre at UCL). Stakeholders also commented on the outcomes specified in the scope, and these were amended accordingly.		
Population size	Approximately 150 people in England would be eligible for treatment with patisiran. *Estimated by the National Amyloidosis Centre.*		
Process (TA/HST)	HST		
Proposed changes to remit (in bold)	None		
Costing implications	The cost of patisiran is not yet known so the overall resource impact cannot currently be estimated. For those people who would use patisiran, there would be some offsetting savings across both the NHS and social care.		
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	Volanesorsen for treating familial chylomicronaemia syndrome		
Topic Selection ID Number	8373	**Wave / Round**	190
HST ID Number	1326		
Company	Akcea Therapeutics		
Anticipated licensing information	***CONFIDENTIAL INFORMATION REMOVED***		
Draft remit	To evaluate the benefits and costs of volanesorsen within its marketing authorisation for treating adults with familial chylomicronaemia syndrome 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 volanesorsen for treating familial chylomicronaemia syndrome (FCS) is appropriate. The proposed remit is not appropriate and should be amended as follows: To evaluate the benefits and costs of volanesorsen within its marketing authorisation for treating ~~**adults with**~~ familial chylomicronaemia syndrome **in adults** for national commissioning by NHS England. This change is editorial only, and does not affect the population in the evaluation. Stakeholders considered that an HST evaluation would be appropriate. Although there is currently no Highly Specialised Service for FCS, treatment is usually managed through the existing lipid clinic network via a small number of tertiary centres. FCS is an umbrella term for specific disorders of lipid metabolism, with a number of potential genetic causes. Stakeholders highlighted that the definition of FCS is based on a combination of clinical symptoms. To ensure treatment is focused on a clinically distinct population, the scope has been amended to specify that consideration should be given to the precise definition and clinical diagnosis of FCS. Stakeholders highlighted that volanesorsen will be used in combination with established management, including fat-restricted diet; this has been incorporated into the scope. They also highlighted the particular impact of FCS on people who are or wish to become pregnant; this has been highlighted in the scope, and if considered by the committee will be considered alongside any corresponding equalities issues.		
Population size	Approximately 55–110 people in England would be eligible for treatment with volanesorsen. Based on estimates from Heart UK and EMA; stakeholders agreed with this estimate.		
Process (TA/HST)	HST		

Proposed changes to remit (in bold)	To evaluate the benefits and costs of volanesorsen within its marketing authorisation for treating ~~**adults with**~~ familial chylomicronaemia syndrome **in adults** for national commissioning by NHS England.
Costing implications	The cost of volanesorsen is not yet known, therefore the resource impact could not be estimated. An increase in drug costs may be offset by reduced treatment costs for recurrent pancreatitis.
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.