National Institute for Health and Care Excellence

Single Technology Appraisal (STA)

Vutrisiran for treating hereditary transthyretin-related amyloidosis [ID5074]

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Comment 1: the draft remit

Section	Consultee/ Commentator	Comments [sic]	Action
Appropriateness	Alnylam Pharmaceuticals	Alnylam regards the proposed evaluation of vutrisiran to be appropriate.	Thank you for your comment. No action required.
	National Amyloidosis Centre, UCL & Royal Free Hospital	Why is this new treatment, with major benefits to patients' convenience and avoidance of steroid pre-meds, not being evaluated as a Highly Specialised Technology Appraisal, as per inotersen and patisiran? It is a very rare disease, and this new treatment is essentially identical to patisiran, but delivered by a much superior 3 monthly sub-cut route in lieu of onerous 3 weekly intravenous infusions that require steroid and other pre-med drugs each time.	Thank you for your comment. NICE considered that vutrisiran did not meet all the criterion listed on Highly Specialised Technology checklist.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	The evaluation and the evaluation route seem appropriate.	Thank you for your comment. No action required.

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Wording	Alnylam Pharmaceuticals	Alnylam regards the wording of the remit to be appropriate.	Thank you for your comment. No action required.
	National Amyloidosis Centre, UCL & Royal Free Hospital	Yes.	Thank you for your comment. No action required.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	Yes.	Thank you for your comment. No action required.
Timing issues	Alnylam Pharmaceuticals	Alnylam sees this evaluation as highly urgent for the NHS. Hereditary transthyretin-mediated (hATTR) amyloidosis is an ultra-rare condition, the natural course of which is characterised by progressive disability and mortality due to disease complications. It is currently managed through an NHS England Highly Specialised Service and a single national treatment centre, the NHS National Amyloidosis Centre (NAC). Patisiran is the most used therapeutic in the UK for patients with hATTR amyloidosis due to its efficacy. Current standard of treatment with patisiran has associated burdens, including: A high-touch, time-consuming intravenous (IV) administration process with frequent dosing (every 3 weeks [Q3W]) with the potential for infusion-related reactions (IRRs) and IV infusion-related complications at the site of the peripheral IV catheter, such as extravasation or phlebitis.	Thank you for your comment. No action required.

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		A premedication regimen with associated adverse effects, required every treatment session to minimise the risk of IRRs. [redacted]Travel by patients across England to the NAC in London for initial treatments, potentially requiring significant travel for patients with hATTR amyloidosis. The summary of product characteristics (SmPC) for patisiran states patisiran can be considered for delivery via homecare after at least 3 well-tolerated infusions in a clinic.[1] Currently, all patients in the UK are moved to homecare for patisiran administration following treatment initiation at the NAC. Alnylam estimates a single treatment session with patisiran can take 3.5 hours not including travel, consisting of: Premedication regimen ≥ 60 mins before patisiran infusion, consisting of an IV corticosteroid (dexamethasone 10 mg or equivalent), H1 blocker (diphenhydramine 50 mg or equivalent), H2 blocker, and oral paracetamol.[1] In the event of a shortage of any component of premedication, treatment would be severely compromised for patients with hATTR amyloidosis. IV infusion which takes approximately 80 mins. Patient monitoring post-infusion which takes approximately 60 mins. In addition, Alnylam estimates that treatment fatigue or drowsiness from premedication can last up to 2 days. Patients are also typically fragile and elderly, amplifying these negative impacts of treatment and potentially decreasing treatment adherence. Furthermore, as noted, patients on patisiran are reliant on a robust homecare delivery service requiring the availability of healthcare professionals (HCP) trained to provide IV infusions. Alnylam notes that homecare delivery services in the UK are experiencing chronic long-term	

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		staff shortages, potentially jeopardising the administration of patisiran to patients in accordance with their ongoing Q3W treatment schedule. Similarly, the NAC has limited infusion capacity for patients with hATTR amyloidosis and is also reliant on robust clinic staffing. Of note, the European Medicines Agency (EMA) Committee for Orphan Drug Medicinal Products (COMP) concluded that for treatment with patisiran, 2–10 hours of active HCP time is lost per patient with hATTR amyloidosis per year.[2] Alnylam notes chronic nurse staffing challenges in the UK NHS particularly as a result of the COVID pandemic[3]; thus, should there be a failure of homecare delivery in the UK, it is unlikely that the NAC could accommodate patisiran infusions for all patients affected. Therefore, there is an urgent need for a therapy that has comparable effectiveness to patisiran but lacks the associated important limitations. Eliminating the burdens of patisiran would benefit patients, their carers, HCPs, and NHS England, and importantly, would minimise the risks of treatment interruption in this progressive and fatal disease. Vutrisiran is an effective therapy for patients with hATTR amyloidosis that will be administered: Every 3 months i.e., 4 times per year as opposed to 17–18 times per year for patisiran. By subcutaneous (SC) injection, which is a less resource- and labour-intensive mode of administration compared to IV administration for patisiran. The introduction of vutrisiran would reduce treatment burden on patients and their carers in addition to decreasing the resource demands on the NHS system. The full extent of benefits vutrisiran would provide are discussed below in more detail in a response to a 'NICE question for consultation', specifically responding to the question posing whether vutrisiran would	

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		produce a substantial impact on health-related benefits, or a 'step-change' in management. Some of these substantial impacts include: Eliminating the risk of IRRs and the need for administration of a premedication regimen associated with IV administration of patisiran, due to the SC route of administration for vutrisiran. Minimising the risk to patients with hATTR amyloidosis of an inability to deliver their treatment, by greatly reducing the treatment burden placed on homecare providers and the NAC in terms of both the frequency of administrations (every quarter vs. every 3 weeks with patisiran) and the time required per administration (less than 5 minutes vs. more than an hour). Contributing to alleviating the pressures faced by the UK healthcare system since vutrisiran can be administered in a variety of outpatient settings, in contrast to the need for a lengthy hospital visit to the NAC or healthcare home visits for IV infusion of patisiran. In the current COVID environment, with an increasing rate of COVID-related hospitalisation in July 2022,[4] the use of vutrisiran may minimise the risk of COVID transmission in the highly vulnerable older age target population by reducing patients' frequency and intensity of healthcare contacts. Furthermore, these benefits would be realized in respect to all iatrogenic infections. The advantages that vutrisiran provides over patisiran to patients with hATTR amyloidosis, their carers, and clinicians would therefore address an urgent unmet need and would be experienced immediately upon patient switch from patisiran to vutrisiran treatment. Recognising the urgent need, Alnylam has been proactive in engaging with NHS England and NICE since 2021 to understand how to best collaborate to ensure the benefits of vutrisiran are realised in England as soon as possible.	

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		Alnylam notes, with great interest, discussion of a 'proportionate approach' by NICE, particularly related to potential pilots for lighter-touch evaluation approaches, where appropriate, to better utilise NICE resources and enable patients to benefit from new therapies more rapidly.[5,6] Alnylam believes it would be rational for such an approach to be applied to vutrisiran, considering the health economics are well understood after the highly specialised technology (HST) appraisal of patisiran for the same population of patients with hATTR amyloidosis in 2018/19,[7] and the comparable clinical efficacy between vutrisiran and patisiran with the aforementioned added benefits vutrisiran provides. Alnylam is very eager to learn more and to participate in a proportionate approach pilot. We would encourage NICE engagement with Alnylam to discuss any plans for pilots, as it would allow patients with hATTR amyloidosis to benefit from vutrisiran sooner than currently proposed appraisal timelines. The Department for Health and Social Care published the UK Rare Disease Framework in 2021. In this framework, four main priorities for patients with rare diseases in the UK are set out, including better coordination of care and improved access to treatment.[8] Making vutrisiran available to patients with hATTR amyloidosis aligns strongly with these priorities and further supports the urgency of this appraisal.	
	National Amyloidosis Centre, UCL & Royal Free Hospital	The new technology is essentially a reformulated version of patisiran with very substantial advantages to patients – simple 3 monthly sub-cut injections vs onerous 3 weekly i.v. infusions requiring pre-medication with steroids and antihistamines etc. It would be a great shame if this new development cannot be implemented urgently into NHS rare disease practice.	Thank you for your comment. No action required.

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	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	This drug may be suitable for patients that are intolerant or not suitable to have patisiran or inotersen. Vutisiran could save their lives. Vutisiran does not require pre-medication (patisiran does), which makes it easier to adminster and reduces side effects from the premedication. It may save money to the NHS since it is administered once every 13 weeks.	Thank you for your comment. No action required.
Any additional comments on the draft remit	Alnylam Pharmaceuticals	NA	Thank you for your comment. No action required.
	National Amyloidosis Centre, UCL & Royal Free Hospital	NA	Thank you for your comment. No action required.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	NA	Thank you for your comment. No action required.
Background information	Alnylam Pharmaceuticals	Commonly used disease progression schemes for patients with hATTR amyloidosis include the familial amyloid polyneuropathy (FAP) staging system and the polyneuropathy disability (PND) scoring system. In the Background section of the draft scope, FAP is used to define levels of neuropathy; however, in the Questions for consultation section, stage 4 polyneuropathy is mentioned, indicating PND score (excluding stage 0, FAP	Thank you for your comment. No action required.

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		staging includes 3 stages [stages 1, 2, and 3], while PND score is more detailed and includes 5 stages [stages 1, 2, 3a, 3b, and 4]). The European SmPC states that vutrisiran is indicated for the treatment of hATTR amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy, defined by FAP stage.	
	National Amyloidosis Centre, UCL & Royal Free Hospital	Should clarify the similarity of new drug with patisiran but emphasising superiority of delivery mechanism, allowing 3 monthly sub-cut administration without hefty steroid and other pre-medication. Need to be clear the new treatment is not an add-on but would be a substitution for current first generation gene silencers, i.e. a second generation treatment.	Thank you for your comment. The background section of the scope aims to provide a brief summary of the disease and how it is managed, it is not designed to be exhaustive in its detail. No changes were made to the scope.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	The background information is true, but it is a simplification. It gives the impression that the main problem for the patient is losing the ability to walk. This is a progressive and devastating disease that can cause constant and severe pain, even at night disturbing sleep; The severe diarrhea often causes social isolation and malnutrition. Loss of hand function causes loss of independence of the patients and severe disability. Urinary symptoms, loss of sexual function, loss of sight and mental deteriroration	Thank you for your comment. The background section of the scope aims to provide a brief summary of the disease and how it is managed, it is not designed to be exhaustive in its detail. More details on the disease and its complications will be discussed during the

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			appraisal. No changes were made to the scope.
Population	Alnylam Pharmaceuticals	Alnylam regards the population definition to be appropriate; however, it should be noted that the European SmPC states that vutrisiran is indicated for the treatment of hATTR amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy.	Thank you for your comment. No action required.
	National Amyloidosis Centre, UCL & Royal Free Hospital	It has become clear that a small proportion of black individuals carrying the TTR V122I gene variant have amyloid polyneuropathy that would be an indication for treatment. Currently, this population are presenting late and a much more patient-friendly administration route may well be advantageous in gaining engagement from the Afro-Caribbean population.	Thank you for your comment. No action required.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	Yes	Thank you for your comment. No action required.
Subgroups	Alnylam Pharmaceuticals	Alnylam regards the population definition without subgroups as appropriate.	Thank you for your comment. No action required.
	National Amyloidosis Centre, UCL & Royal Free Hospital	Note above.	Thank you for your comment. No action required.

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	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	NA	Thank you for your comment. No action required.
Comparators	Alnylam Pharmaceuticals	*Are inotersen and patisiran relevant comparators?* *Which treatments are considered to be established clinical practice in the NHS for hATTR amyloidosis?* In accordance with section 1.2 of the NICE procedure note for the draft scope, Alnylam considers that, for the evaluation to be most efficient, the only relevant comparator for vutrisiran is patisiran. While both patisiran and inotersen are recommended by NICE, patisiran is the current standard of care for patients with hATTR amyloidosis in England and it alone constitutes established clinical practice. Based on Alnylam's understanding of UK clinical practice at the NAC and reflected in product market shares, patisiran is currently utilized in all treatment-eligible patients with hATTR amyloidosis as the first-choice therapy. Inotersen use is reserved for exceptional circumstances only for patients who are unable to receive patisiran for non-clinical reasons, for example due to homecare availability challenges. Thus, inotersen does not occupy the same positioning in the treatment pathway as patisiran in England. Additionally, NAC clinicians [redacted] and [redacted]	Thank you for your comment. At the scoping stage of the appraisal, identification of comparators should be inclusive. The potential comparators listed in the scope represent treatments used to treat hereditary transthyretin-related amyloidosis in NHS clinical practice. Any exclusion from the decision problem in the company submission should be fully justified and will be considered during the course of the appraisal.

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		[redacted], have informed Alnylam that they foresee vutrisiran replacing patisiran as the standard of care for patients with hATTR amyloidosis in the UK. Based on prior communication in January 2022 with [redacted] This highlights the increasing irrelevance of inotersen since it is plausible that no patients in England, or very few future patients in England, will be receiving inotersen in the near future. Alnylam also notes in HST9 for inotersen, the committee stated that:[11] "Clinical trial evidence shows that inotersen slows progression of the disease considerably, although its long-term benefits are uncertain." In contrast, in HST10 for patisiran, the committee stated that:[7] "Clinical trial evidence shows that patisiran reduces disability and improves quality of life, by enabling patients to return to work, carry out daily activities, participate in a more active family and social life, and maintain their independence and dignity. There is also evidence suggesting that patisiran may provide long-term benefits by stopping the progression of amyloidosis and potentially reversing it." Alnylam also notes contraindications exclusive to inotersen include: platelet count < 100 x 10^9/L prior to treatment; urine protein to creatinine ratio (UPCR) ≥ 113 mg/mmol (1 g/g) prior to treatment; estimated glomerular	

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		filtration rate (eGFR) < 45 ml/min/1.73 m^2 [moderate to severe loss of kidney function]; and severe hepatic impairment.[12] It is important to note that the single listed contraindication for vutrisiran is identical to that of patisiran, namely hypersensitivity to the active ingredient or excipients.[1,10] Of note, the EMA COMP noted:[2] "In contrast, the SmPC for vutrisiran does not have these contraindications [of inotersen], thus broadening the patient population addressable by vutrisiran compared to inotersen. Vutrisiran SmPC also does not require monitoring since there were no effects seen on platelet counts or any evidence of renal toxicity in the HELIOS-A study, which included 4 patients with eGFR 30-45 mL/min/1.73m^2 at baseline [i.e., patients who would be ineligible for treatment with inotersen per its labelled contraindications]." *Are other experimental treatments such as doxycycline plus tauroursodeoxycholic acid comparators?* Experimental treatments, including doxycycline plus tauroursodeoxycholic acid, are not relevant comparators for inclusion in the appraisal because none of these treatments are commonly used or part of the pathway of care in the UK for patients with hATTR amyloidosis with polyneuropathy. In the appraisal of inotersen, experts from the NAC confirmed that doxycycline plus tauroursodeoxycholic acid is not used in the UK, and thus these were explicitly excluded from the scope.[13] Given their irrelevance even back in 2018, Alnylam sees these experimental treatments even more irrelevant now in 2022 when other authorized treatments are available. *Are there any other treatments that should be included as comparators?* Alnylam does not regard any other treatment options besides patisiran as relevant comparators. The evidence base for vutrisiran also supports the	.

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		relevance of the comparison versus patisiran, since the HELIOS-A trial included both of these small interfering RNA therapies.	
	National Amyloidosis Centre, UCL & Royal Free Hospital	Yes, but note that diflunisal is not funded at present and not easy to access.	Thank you for your comment. No action required.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	Patisiran and inotersen seem to be the correct comparators. All other drugs mentioned are not comparators, in our view.	Thank you for your comment. No action required.
Outcomes	Alnylam Pharmaceuticals	Alnylam regards the outcome measures proposed as appropriate. *Is serum transthyretin a relevant outcome?* Serum transthyretin (TTR) is a relevant outcome measure for trials in hATTR amyloidosis. Transthyretin is a tetrameric protein composed of four monomers.[14] In the case of transthyretin-mediated amyloidosis, the tetrameric protein destabilises into unstable monomers and TTR fragments that can misfold and form amyloid fibril deposits in multiple organs, including the peripheral nervous system, heart, and GI tract, leading to cellular injury and organ dysfunction with corresponding clinical manifestations.[14-17] The patisiran SmPC and European vutrisiran SmPC highlight that the mechanism of action for both patisiran and vutrisiran is reducing serum and tissue TTR levels through mRNA interference pathways in the liver.[1,10] Additionally, in HST10 for patisiran, it was noted by NICE that clinical experts agree that the stopping or reversing amyloid deposition and decreasing subsequent neuropathy is dependent on the reduction of serum	Thank you for your comment. No action required.

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		TTR.[7] Evidence from the HELIOS-A trial showed a rapid and sustained reduction in serum TTR levels in patients treated with vutrisiran, noninferior to within-study patisiran as demonstrated by a prespecified secondary endpoint analysis at Month 18.[18] Therefore, serum TTR is a relevant outcome for trials in hATTR amyloidosis. However, due to a potential lack of assay reagent availability, assay variability, and intra-patient variability in peak to trough serum TTR levels at any given point in time, serum TTR levels may have less relevance as an outcome measure in routine clinical practice. *Which elements of autonomic function are affected by the condition and might be improved by vutrisiran?* hATTR amyloidosis is a multisystem disease with heterogeneous clinical presentation that includes sensory, motor, and autonomic (e.g., diarrhea, sexual dysfunction, orthostatic intolerance) polyneuropathy and cardiomyopathy, with the potential involvement of other organ systems as well.[14,17,19,20] The primary endpoint of the HELIOS-A study was based on mNIS+7,[18] which assesses the progression of the motor and the sensory aspects of polyneuropathy, as well as some autonomic manifestations, such as postural hypotension, and correlates with both FAP and PND scores. Autonomic assessment from mNIS+7 at Month 9 and 18 of the HELIOS-A trial demonstrated improvement in autonomic nerve function for patients on vutrisiran compared to placebo.[21,22] The Norfolk Quality of Life – Diabetic Neuropathy (QoL-DN) questionnaire was a secondary endpoint in the HELIOS-A trial.[18] One of the 5 domains of the Norfolk QoL-DN questionnaire assesses symptoms of autonomic nerve dysfunction and their impact on activities of daily living. Significant improvements in this domain of the Norfolk QoL-DN questionnaire were observed in vutrisiran-treated patients when compared to placebo in the HELIOS-A trial.[21]	

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		Modified body mass index (mBMI) was also a secondary endpoint in the HELIOS-A trial.[18] mBMI (BMI multiplied by serum albumin concentration [g/L]) is understood to be an important indicator of autonomic gastrointestinal nerve function.[23] Further enhancements in autonomic function were demonstrated by significantly improved mBMI measures in vutrisiran-treated patients when compared to placebo in the HELIOS-A trial.[21] Therefore, all relevant data indicate that vutrisiran improves autonomic function in patients affected by hATTR amyloidosis. Data from the APOLLO trial for patisiran also demonstrated significant improvement in mBMI and improvements in the autonomic domain of the Norfolk QoL-DN questionnaire in patients with hATTR amyloidosis treated with patisiran compared to placebo, demonstrating similarities in autonomic improvement for vutrisiran and patisiran.[24,25] *Would vutrisiran have an effect on cardiomyopathy outcomes?* The effects of vutrisiran on patients with transthyretin amyloidosis (ATTR) with cardiomyopathy are currently being explored in the ongoing phase 3, randomised, double-blind, placebo-controlled, multicenter study, HELIOS-B.[26] Alnylam does not consider that cardiomyopathy measures are relevant to include in the present scope for patients with hATTR amyloidosis with polyneuropathy considering Alnylam is proposing a cost comparison submission against patisiran which is approved to treat patients with hATTR amyloidosis with polyneuropathy. Please see below response to 'NICE question for consultation', specifically regarding the appropriateness of using cost-comparison methodology for vutrisiran.	
	National Amyloidosis Centre, UCL &	Need to consider the benefit to patients related to 3 monthly sc vs current much more onerous 3 weekly infusions.	Thank you for your comment. No action required.

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	Royal Free Hospital		
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	Yes. The modified mNIS + 7 (mNIS + 7) scale was specifically designed to assess polyneuropathy impairment in patients with hATTR amyloidosis. The autonomic symptoms of the disease are very intrusive and often understimated.	Thank you for your comment. No action required.
Equality and diversity	Alnylam Pharmaceuticals	Alnylam does not consider that the draft remit and scope need to be modified to meet equality goals.	Thank you for your comment. No action required.
	National Amyloidosis Centre, UCL & Royal Free Hospital	NA	Thank you for your comment. No action required.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	The population is defined as: “People with hereditary transthyretin-related amyloidosis” There are over 100 mutations of the TTR gene, resulting in different presentations of the disease. Caucasian patients tend to have some mutations that result in more neuropathy and less cardiomyopathy (eg V30M), while patients from the black community tend to develop more cardiomyopathy and fewer neurological symptoms (eg V122I). Will patients from the black community be disadvantaged?	Thank you for your comment. The appraisal committee will consider the impact of its recommendations on protected characteristics as stated in equality legislation during the appraisal. No action required.

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		Patients with more advanced disability or those who live in remote areas have significant difficulties travelling for treatment. A treatment that minimises inconvenience has a higher value for these patients. It would be ideal if the treatment could be home delivered to the patients, in the same way	
Other considerations	Alnylam Pharmaceuticals	Alnylam does not have any additional issues to suggest.	Thank you for your comment. No action required
	National Amyloidosis Centre, UCL & Royal Free Hospital	NA	Thank you for your comment. No action required.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	The two drugs that are currently available have many positive benefits which as patients have been appreciated. Vutisiran, that is administered by injection every 13 weeks is a step change that is proving to be positive compared with Patisiran and Inoterson. Patisiran is administered every 3 weeks with a nurse visiting the patient's home for three hours at a time. This includes giving the patient a variety of drugs which depending on the patient, does/can have adverse effects. Moving to an injection every 13 weeks will reduce the cost and demands of the nursing staff providing the treatment. Inoterson is a weekly injection that is self- administered which some patients have issues with doing. Added to this a nurse visits the patient every two weeks to take bloods for evaluation that again will be high in cost and nurse time.	Thank you for your comment. No action required.
Questions for consultation	Alnylam Pharmaceuticals	NA	Thank you for your comment. No action required.

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	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	NA	Thank you for your comment. No action required.
Additional comments on the draft scope	Alnylam Pharmaceuticals	Alnylam would welcome a scoping workshop with clinicians present to further discuss NICE's view on the appropriateness of our current plan for a vutrisiran cost comparison submission against patisiran. Alnylam is fully prepared to make a cost comparison submission in late Q3 or early Q4 2022, and as previously raised with the scoping team, is keen to discuss the possibility to expedite the appraisal from currently communicated timelines for the benefit of patients, carers, HCPs, and NHS England. As noted in this document, Alnylam is greatly interested in the 'proportionate approach' being considered by NICE, particularly for potential pilots for lighter-touch evaluations where appropriate.5,6 Again, we believe it would be rational to apply such an approach to vutrisiran considering that the health economics are well understood and considering an HST appraisal of patisiran was performed for the same population of patients with hATTR amyloidosis in 2018/19.7 This is also supported by the comparable clinical efficacy between vutrisiran and patisiran with the added benefits vutrisiran offers. Alnylam is very eager to learn more and to participate in a proportionate approach pilot and would encourage NICE to engage with Alnylam to discuss any plans for pilots, if it would allow patients to benefit from vutrisiran sooner than currently proposed appraisal timelines.	Thank you for your comment. No action required.
	National Amyloidosis	It is very difficult to understand why NICE is not conducting this new therapy as a HST appraisal.	Thank you for your comment. NICE

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	Centre, UCL & Royal Free Hospital		considered that vutrisiran did not meet all the criterion listed on Highly Specialised Technology checklist.
	(UKATPA) and The UK Amyloidosis advisory Group (UKAAG)	The aproval of patisiran and inotersen represented a huge step ahead for ATTR amyloidosis patients. The approval of vutisiran would represent another very significant step in the treatment of this devastating disease.	Thank you for your comment. No action required.