

vutrisiran sodium solution for injection in prefilled syringe (Amvuttra®)

Alnylam UK Ltd

4 September 2026

The Scottish Medicines Consortium (SMC) has completed its assessment of the above product and, following review by the SMC executive, advises NHS Boards and Area Drug and Therapeutics Committees (ADTCs) on its use in NHSScotland. The advice is summarised as follows:

ADVICE: following a full submission

vutrisiran sodium (Amvuttra®) is accepted for restricted use within NHSScotland.

Indication under review: for the treatment of wild-type or hereditary transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR-CM).

SMC restriction: as monotherapy for the treatment of adult patients with wild-type or hereditary ATTR-CM.

In a phase III study, treatment with vutrisiran monotherapy significantly reduced the risk of all-cause mortality and recurrent cardiovascular events compared with placebo in patients with wild-type or hereditary ATTR-CM. There are limited data to support an indirect comparison of vutrisiran and tafamidis.

This advice applies only in the context of an approved NHSScotland Patient Access Scheme (PAS) arrangement delivering the cost-effectiveness results upon which the decision was based, or a PAS/ list price that is equivalent or lower.

Chair
Scottish Medicines Consortium

1. Clinical Context

1.1. Medicine background

Vutrisiran sodium (hereafter referred to as vutrisiran) is a small interfering ribonucleic acid (siRNA) therapy that targets and suppresses the production of both wild-type and variant transthyretin (TTR) protein in the liver, which are the pathogenic proteins causing TTR amyloidosis (ATTR). By reducing circulating TTR levels through RNA interference, vutrisiran decreases TTR amyloid deposition in tissues.^{1, 2}

The recommended dose of vutrisiran is 25 mg administered by subcutaneous injection once every 3 months. Vitamin A supplementation at approximately, but not exceeding, 2,500 to 3,000 international units vitamin A per day is advised for patients treated with vutrisiran. Treatment should be started as early as possible in the disease course to prevent the accumulation of disability.¹ Refer to the Summary of Product Characteristics (SPC) for further information.

1.2. Disease background

Transthyretin amyloidosis (ATTR) is a form of systemic amyloidosis resulting from the production of TTR proteins in the liver. These accumulate as amyloid deposits (amyloidosis) in tissues around the body, particularly the peripheral nerves (causing polyneuropathy), the heart and gastrointestinal tract. The accumulation of TTR amyloid deposits in the heart results in transthyretin amyloidosis with cardiomyopathy (ATTR-CM), a rare and fatal condition which causes the heart to stiffen resulting in heart failure symptoms. ATTR-CM can be wild-type when the TTR proteins become structurally unstable with age and this mainly occurs in older patients (usually >60 years, with a reported average age of onset of 75 years), affects more men than women and median survival is estimated to be around 3.5 years. ATTR-CM can also be hereditary (also referred to as variant ATTR-CM), caused by one of over 120 mutations in the TTR gene. Patients with hereditary disease are generally younger than those with wild-type disease but age varies with the mutation (age of onset reported as varying between 30 and 80 years) and the median survival is thought to be lower, around 2.5 years. ATTR-CM causes the heart to stiffen resulting in heart failure symptoms.^{2, 3}

1.3. Company proposed positioning

The submitting company has requested that vutrisiran is restricted for use as monotherapy for the treatment of adult patients with wild-type or hereditary ATTR-CM.

1.4. Treatment pathway and relevant comparators

Tafamidis (a stabiliser of TTR) is the only treatment accepted for use in NHSScotland for the treatment of wild-type and hereditary ATTR-CM (SMC2585). Historically, treatment has been directed at symptom management such as diuretics for congestive symptoms and antiarrhythmic drugs, pacemakers and automatic implantable cardioverter defibrillators for arrhythmias and conduction defects. For some patients, cardiac and/or liver transplant might be an option. However, given the generally advanced age of patients with ATTR-CM at diagnosis, and their co-morbid burden of illness, transplantation is rarely an option.^{2, 4}

2. Summary of Clinical Evidence

2.1. Evidence for the licensed indication under review

Evidence to support the efficacy and safety of vutrisiran comes from the HELIOS-B study.^{2, 5} Details are presented in Table 2.1.

Table 2.1. Overview of relevant study

Criteria	HELIOS-B ^{2, 5}
Study design	International, multicentre, randomised, double-blind, placebo-controlled, phase III study.
Eligible patients	• 18 to 85 years of age • hATTR with cardiomyopathy or wtATTR with cardiomyopathy • History of heart failure with at least one prior hospitalisation for heart failure (not due to arrhythmia or a conduction system disturbance treated with a permanent pacemaker) or clinical evidence of heart failure (with or without hospitalisation) manifested by signs and symptoms of volume overload or elevated intracardiac pressures that requires treatment with a diuretic • Meets one of the following criteria: • Tafamidis naïve and not planning to commence treatment with tafamidis during the first 12 months following randomisation, or • on tafamidis per an approved cardiomyopathy indication. • Screening NT-proBNP >300 nanograms/L and <8,500 nanograms/L; in patients with permanent or persistent atrial fibrillation, screening NT-proBNP >600 nanograms/L and <8,500 nanograms/L • Able to complete ≥150 metres on the 6MWT at screening • Karnofsky performance status of ≥60% • Does not have primary or leptomeningeal amyloidosis; NYHA class IV heart failure or NYHA class III heart failure with stage III ATTR (defined as NT-proBNP >3,000 nanograms/L and eGFR <45 ml/min); or polyneuropathy disability score III or IV.
Treatments	Patients were randomised to receive vutrisiran 25 mg by subcutaneous injection or placebo every 12 weeks for up to 36 months. Patients who were on tafamidis at baseline were encouraged, if considered medically appropriate by the investigator, to remain on tafamidis for the duration of the study. Initiation of tafamidis in previously naïve patients during the study was allowed in countries where tafamidis was approved. All patients were encouraged to take the recommended daily allowance of vitamin A for the duration of their participation in the study while being administered the study medicine.
Randomisation	Patients were randomised equally. Randomisation was stratified according to baseline tafamidis use (yes versus no), ATTR amyloidosis disease type (hATTR versus wtATTR amyloidosis) and NYHA (NYHA class I versus II) and age (<75 versus ≥75 years).
Co-primary outcomes	Composite of all-cause mortality and recurrent cardiovascular events (defined as hospitalisations for cardiovascular causes or urgent visits for heart failure) during the double-blind period (up to 36 months), assessed in (1) the overall population and (2) vutrisiran monotherapy subgroup.
Secondary outcomes	• Change from baseline in 6MWT • Change from baseline in the KCCQ-OS • All-cause mortality • Change from baseline in NYHA class.

Statistical analysis	Efficacy analyses were performed in the FAS population, which included all randomised patients who received any amount of study medicine and the vutrisiran monotherapy FAS, which included all randomised patients who were tafamidis naïve at baseline and received any amount of study medicine. If the co-primary outcomes were statistically significant, a hierarchical statistical testing strategy was applied for the key secondary outcomes in the order detailed above for both the vutrisiran monotherapy FAS and overall population FAS, with testing progressing only if both were significant.
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Abbreviations: ATTR = transthyretin amyloidosis; 6MWT = 6-minute walk test; FAS = full analysis set; hATTR = hereditary transthyretin amyloidosis; KCCQ-OS = Kansas City Cardiomyopathy Questionnaire Overall Summary Score; NT-proBNP = N-terminal pro B-type natriuretic peptide; NYHA = New York Heart Association Functional Classification; wtATTR = wild-type transthyretin amyloidosis

At the primary analysis (08 May 2024), vutrisiran was associated with statistically significant reductions in the overall population and the vutrisiran monotherapy subgroup for the primary composite outcome, all-cause mortality and recurrent cardiovascular (CV) events, compared with placebo and significant benefits for the secondary outcomes.^{2, 5} See Table 2.2 for further details.

Table 2.2: Primary and key secondary outcomes from the HELIOS-B study at the primary analysis (08 May 2024).^{2, 5}

	Overall population (FAS)		Vutrisiran monotherapy subgroup	
	Vutrisiran (n=326)	Placebo (n=328)	Vutrisiran (n=196)	Placebo (n=199)
Primary outcome: composite of all-cause mortality and recurrent CV events up to 36 months				
Patients with at least one event, n (%)	125 (38%)	159 (48%)	76 (39%)	105 (53%)
All-cause mortality, n (%)	51 (16%)	69 (21%)	36 (18%)	46 (23%)
CV events, n (%)	112 (34%)	133 (41%)	66 (34%)	87 (44%)
HR (95% CI), p-value	0.72 (0.56 to 0.93), p=0.01		0.67 (0.49 to 0.93), p=0.02	
Key secondary outcomes				
LS mean change in 6MWT from baseline at 30 months, metres	-45.4	-71.9	-59.7	-91.8
LS mean difference (95% CI), p-value	26.5 (13.4 to 39.6), p<0.001		32.1 (14.0 to 50.2), p<0.001	
LS mean change from baseline in KCCQ-OS score at 30 months, points	-9.7	-15.5	-10.8	-19.5
LS mean difference (95% CI), p-value	5.8 (2.4 to 9.2), p<0.001		8.7 (4.0 to 13.4), p<0.001	
All-cause mortality at 42 months, n (%)	60 (18%)	85 (26%)	43 (22%)	58 (29%)
HR (95% CI), p-value	0.65 (0.46 to 0.90), p=0.01		0.66 (0.44 to 0.97), p=0.045	
Improved or stable NYHA class at 30 months, %	68%	61%	66%	56%
Difference (95% CI), p-value	8.7% (1.3 to 16), p=0.02		12% (2.7 to 22), p=0.01	

Abbreviations: 6MWT = 6-minute walk test; CI = confidence interval; CV = cardiovascular; FAS = full analysis set; HR = hazard ratio; KCCQ-OS = Kansas City Cardiomyopathy Questionnaire Overall Summary Score; LS = least squares; NYHA = New York Heart Association Functional Classification.

Treatment with vutrisiran was associated with a reduction in risk of the exploratory outcome, time to first CV event or all-cause mortality compared with placebo, in both the overall population (HR: 0.72, 95% CI = 0.57 to 0.91) and the vutrisiran monotherapy subgroup (HR: 0.64, 95% CI = 0.48 to 0.87). A regulator considered this analysis to support the robustness and assumption of clinical relevance of the primary analysis in both populations.²

Initiation of tafamidis in previously naïve patients during the study was allowed. Similar numbers in both arms of the vutrisiran monotherapy subgroup initiated tafamidis after randomisation (n=44 vutrisiran, n=41 placebo). A post hoc analysis censored observations after tafamidis initiation and found results consistent with the original vutrisiran monotherapy subgroup analysis.⁶

The submitting company conducted a post hoc in-study comparison of patients receiving vutrisiran monotherapy (n=196) and patients receiving tafamidis monotherapy (patients in placebo group of the overall population who were on background tafamidis at baseline, n=129). Results of the in-trial comparison were used to inform the economic model. Stabilised inverse probability of treatment weighting (IPTW) was used to balance baseline differences between the vutrisiran and tafamidis monotherapy groups, given these groups were not randomised within HELIOS-B. In the composite of all-cause mortality and recurrent CV events up to 36 months, there was no difference in efficacy between vutrisiran monotherapy and tafamidis monotherapy (HR: 0.83, 95% CI: 0.54 to 1.29). Similarly, for other outcomes used to inform the economic model (all-cause mortality up to 42 months, recurrent CV events, change in NYHA class and change in EQ-5D-5L), there was no evidence of a difference between vutrisiran monotherapy and tafamidis monotherapy.⁷

2.2. Health-related quality of life outcomes

Health-related quality of life was assessed using three questionnaires: Kansas City Cardiomyopathy Questionnaire Overall Summary (KCCQ-OS), EQ-5D-5L and Norfolk Quality of Life-Diabetic Neuropathy (Norfolk QoL-DN).⁵

Vutrisiran was associated with benefits in KCCQ-OS, a key secondary outcome detailed in Table 2.2. At month 30, vutrisiran, compared with placebo, appeared to improve change from baseline in EQ-5D-5L index score and visual analogue scale score. At month 30, vutrisiran appeared to improve neuropathy-related quality of life as assessed by Norfolk QoL-DN compared with placebo in the overall population, with a larger benefit observed in patients receiving vutrisiran monotherapy and smaller in those also receiving tafamidis.²

2.3. Supportive studies

The submitting company provided supporting data from the ongoing open-label extension (OLE) period of HELIOS-B. Patients who had completed the double-blind period were eligible to enter the OLE for up to 2 years and receive vutrisiran 25 mg by subcutaneous injection every 3 months.

Of 478 patients who completed double-blinded treatment, 466 (97%) entered the OLE period, with 213 patients in the vutrisiran group and 189 patients in the placebo group continuing

treatment at the month 12 OLE data cutoff. Reduced risk of time to first CV event or all-cause mortality persisted through 12 months of OLE treatment in those previously treated with vutrisiran in the overall population (HR = 0.63, 95% CI: 0.51 to 0.79) and the monotherapy population (HR = 0.58, 95% CI: 0.44 to 0.76). Results also suggested a continued benefit of vutrisiran on time to all-cause mortality, quality of life measures and cardiac biomarkers.⁸

2.4. Indirect evidence to support clinical and cost-effectiveness comparisons

In the absence of direct evidence comparing vutrisiran with tafamidis, an exploratory unanchored matching adjusted indirect treatment comparison (MAIC) was conducted using data from HELIOS-B and ATTR-ACT.^{5,9} The exploratory MAIC was not used in the economic base case but informed scenario analyses.

[Other data were also assessed but remain confidential.](#)*

3. Summary of Safety Evidence

Evidence from the HELIOS-B study at the primary analysis cut off (08 May 2024), supports the relative safety of vutrisiran compared with placebo for the indication under review. However, placebo is not considered a relevant comparator in this submission. The median duration of treatment in the vutrisiran group was 35.8 months and 33.8 months in the placebo group.²

In the overall population, the incidence of adverse events (AEs) was similar in the two groups; 99% (322/326) in the vutrisiran group and 98% (323/328) in the placebo group had at least one AE, and these were considered related to study medicine in 13% versus 14%, respectively. Serious AEs occurred in 62% in the vutrisiran group and in 67% in the placebo group and these were considered related to study medicine in 0.6% versus 0.3%. AEs leading to discontinuation of vutrisiran or placebo occurred in 3.1% and 4.0%, respectively.²

The most frequently reported serious AEs in either treatment group were cardiac failure (12% versus 17%), atrial fibrillation (8.0% versus 6.1%) and acute cardiac failure (4.0% versus 5.5%).²

Overall, the safety profile of vutrisiran for this indication was consistent with the known safety and no new safety concerns were identified. Ophthalmological assessment is recommended if patients develop ocular symptoms suggestive of vitamin A deficiency.^{1,2}

4. Summary of Clinical Effectiveness Considerations

4.1. Key strengths

- At the primary analysis of the phase III study, HELIOS-B, vutrisiran was associated with statistically significant and clinically meaningful reductions in the composite primary outcome, all-cause mortality and recurrent CV events, compared with placebo in the overall population and the vutrisiran monotherapy subgroup.⁵
- The primary outcome was supported by statistically significant improvements in key secondary outcomes including 6MWT, KCCQ-OS, all-cause mortality, New York Heart Association Functional Classification (NYHA) class and reductions in TTR.⁵

- Supportive OLE data suggest continued benefit through 12 months of open-label follow-up.⁸

4.2. Key uncertainties

- There are limited comparative data to support an indirect comparison of vutrisiran versus tafamidis, the most relevant comparator in Scotland. The submitting company concluded that a robust indirect treatment comparison of the HELIOS-B and ATTR-ACT studies was not feasible due to substantial differences between the studies, including non-contemporaneous populations, differences in baseline characteristics and supportive care, and lack of comparability between placebo arms.^{5, 9} The submitting company also conducted an in-study comparison of vutrisiran monotherapy with tafamidis monotherapy, which it considered to be the best available evidence because it was not possible to conduct an unbiased indirect treatment comparison using data from HELIOS-B and ATTR-ACT. However, this analysis was not pre-specified and is also subject to potential bias, as patients in the tafamidis group had received tafamidis before study entry and were therefore likely already deriving treatment benefit at baseline.
- There is uncertainty regarding the efficacy of vutrisiran in patients with hereditary ATTR-CM, as these patients comprised only 12% of the study population and subgroup analyses suggested potentially lower efficacy for the primary composite outcome (HR: 0.92, 95% CI: 0.49 to 1.72) than that observed in patients with wild-type ATTR-CM (HR: 0.67, 95% CI: 0.51 to 0.90). This uncertainty was particularly pronounced in patients receiving background tafamidis.⁵ However, the hereditary ATTR-CM subgroup was small, therefore results should be interpreted with caution.²
- HELIOS-B represented a mild to moderate patient population; therefore, data are limited regarding efficacy and safety in patients at an advanced stage of the disease.²
- There is uncertainty regarding the place in therapy of vutrisiran in patients already receiving tafamidis. HELIOS-B included some patients receiving tafamidis at baseline; however, the study was not designed to establish the benefit of adding vutrisiran to tafamidis. Furthermore, assessment of vutrisiran in combination with tafamidis is outside the scope of this submission.

4.3. Clinical expert input

Clinical experts consulted by SMC consider vutrisiran to be a therapeutic advancement due to its novel mechanism of action. They also consider that it offers an effective alternative treatment option to tafamidis and can be administered via a different route, which may be preferred by some patients.

4.4. Service implications

No substantial service implications are expected.

5. Summary of Patient and Carer Involvement

The following information reflects the views of the specified Patient Groups.

- We received patient group submissions from Amyloidosis UK and Cardiomyopathy UK, which are both registered charities.
- Amyloidosis UK has received 57% pharmaceutical company funding in the past two years, including from the submitting company. Cardiomyopathy UK has received 10.5% pharmaceutical company funding in the past two years, including from the submitting company.
- ATTR-CM is a progressive, debilitating and ultimately fatal condition that affects every aspect of a patient's life – physical, financial, social, emotional and psychological. Many people experience years of symptoms, including fatigue, breathlessness, swelling and mobility problems, before receiving a diagnosis. During this time, irreversible damage to the heart can occur, substantially affecting quality of life for individuals and their families. Patients' families and carers frequently adjust their own lives by reducing work, retiring early or taking on full care-giving roles as the patient's needs increase.
- It is important that people are given a choice in treatments, which are most suitable for their personal circumstances and their medical condition. Tafamidis is currently the only disease-modifying treatment available in Scotland. Not every patient responds to or tolerates it.
- Vutrisiran would give clinicians and patients a genuine second option. It is the first long-acting, disease-modifying therapy and is administered as a subcutaneous injection, usually every three months. This means that eligible patients could have access to a treatment that fits more easily around everyday life.
- ATTR-CM disproportionately affects people over 60 (wild-type disease) and people of West African ancestry (the V122I hereditary variant), both groups are currently underserved relative to the impact of the condition on them.

6. Summary of Comparative Health Economic Evidence

6.1. Economic case

An economic case was provided by the submitting company and is summarised in Table 6.1.

Table 6.1 Description of economic analysis

Criteria	Overview
Analysis type	Cost-utility analysis.
Time horizon	24 years.
Population	Adult patients with wild-type or hereditary ATTR-CM.
Comparators	The comparator was tafamidis. Following discontinuation of either vutrisiran or tafamidis patients were assumed to receive BSC.

Model description	Markov model with four health states and death. The health states used the NYHA classification to measure the severity of the disease, from NYHA I to NYHA IV. Patients in health states NYHA I, II and III could transition between any health state or death. Patients in the most severe health state NYHA IV either remained in the same state or transitioned to death.
Clinical data	The HELIOS-B study was the primary source of clinical data.⁵ The efficacy, survival and AE incidence rates for patients receiving different treatment in the model were informed by different study subgroups: • Vutrisiran – patients in the vutrisiran arm of the HELIOS-B monotherapy population (no background tafamidis) • Tafamidis – patients in the placebo arm of the overall HELIOS-B population who were receiving background tafamidis • BSC / discontinuation – patients in the placebo arm of the HELIOS-B monotherapy population (no background tafamidis) For treatment discontinuation, data were taken from HELIOS-B vutrisiran discontinuation rates and assumed equal for tafamidis.
Extrapolation	HELIOS-B survival data were only available up to 42 months. Beyond this, Kaplan-Meier (KM) curves were sourced from the HELIOS-B vutrisiran monotherapy and placebo overall arms and informed long-term independent parametric modelling for the vutrisiran and tafamidis treatment arms. In the base case, the loglogistic function was applied in the vutrisiran arm, and the lognormal function was applied for tafamidis. Transition probabilities were sourced from 30 months of collected HELIOS-B data and were applied for the first 10 cycles in the economic model. Beyond this, the mean transition probabilities calculated from observed data between 18-30 months in HELIOS-B were carried forward. Non-fatal treatment discontinuation KM data were sourced from HELIOS-B and parametric functions were fit against these. These were assumed to be the same between vutrisiran and tafamidis. The gamma curve was used in the base case. Treatment waning of 12 months was applied after discontinuing vutrisiran. No waning was applied for tafamidis.
Quality of life	Health state specific utility data were sourced from HELIOS-B. The submitting company considered the estimated values commercial in confidence, and so they cannot be reported. Disutilities were also included to account for transient events such as cardiovascular-related hospitalisations and urgent heart failure visits and AE disutilities. These were sourced from HELIOS-B in the first instance and from the literature. In scenario analysis, the effect of caregiver disutilities were explored. These values were sourced from the literature.
Costs and resource use	Medicine acquisition costs were included for vutrisiran, tafamidis and other background costs for the management of cardiac symptoms. Healthcare resource use costs were included for hospitalisation due to transient events, non-hospitalisation, AE management and terminal care. In scenario analysis for a societal perspective, productivity loss costs were included for patients and caregivers.
PAS/contract price	A Patient Access Scheme (PAS) was submitted by the company and assessed by the Patient Access Scheme Assessment Group (PASAG) as acceptable for implementation in NHSScotland. Under the PAS, a discount of was offered on the list price. A PAS discount is in place for tafamidis and this was included in the results used for decision-making by using estimates of the comparator PAS price.

6.2. Results

The economic evaluation compared the cost and health implications of treatment using either vutrisiran or tafamidis.

SMC considered results for decision-making that took into account all relevant discounts. SMC is unable to present these results due to competition law issues.

[Other data were also assessed but remain confidential.](#)*

6.3. Sensitivity analyses

A range of sensitivity and scenario analyses were considered and descriptions of these key scenarios are provided in Table 6.3.

Table 6.3. Scenario analyses results

	Parameter	Base case	Scenario	Inc. ICER (£/QALY)
	Base case			CIC
1a	Time horizon	24 years	10 years	CIC
1b			20 years	CIC
2a	Perspective	NHS	Societal – caregiver disutilities included	CIC
2b			Societal – caregiver disutilities and costs included	CIC
2c			Societal – caregiver disutilities and costs, patient productivity loss included	CIC
3	Survival analysis modelling	Study-based individual parametric curves	MAIC vutrisiran vs tafamidis HR [Value CiC]	CIC
4a	Parametric survival extrapolation - vutrisiran	Loglogistic	Weibull	CIC
4b			Gamma	CIC
4c			Exponential	CIC
5a	Parametric survival extrapolation - tafamidis	Lognormal	Loglogistic	CIC
5b			Gamma	CIC
5c			Gompertz	CIC
6a	Per-cycle transient event incidence rates NYHA class IV	NYHA IV is 3x more than NYHA III	NYHA IV is equal to NYHA III	CIC
6b			NYHA IV is 4x more than NYHA III	CIC
6c			NYHA IV is 5x more than NYHA III	CIC

Abbreviations: CIC, commercial in confidence; HR, hazard ratio; ICER, incremental cost-effectiveness ratio; Inc., incremental; LY, life year; MAIC, matching adjusted indirect comparison; NHS, National Health Service; NYHA, New York Health Association; QALY, quality adjusted life year

6.4. Key strengths

- The model was well-conducted, with appropriate and comprehensive methods applied.
- The company derived most clinical inputs from the HELIOS-B study, which is relevant to the patient population. Only a few inputs were sourced from the literature where necessary.

6.5. Key uncertainties

- The use of lognormal curve to model tafamidis survival outcomes may be considered favourable. Sensitivity analysis suggests that worse survival outcomes for tafamidis produce upwards impact on results.
- The submitting company used an unplanned in-study comparison of vutrisiran monotherapy with tafamidis monotherapy, as they considered that it was not possible to conduct an unbiased indirect treatment comparison.

7. Conclusion

After considering all the available evidence, the Committee accepted vutrisiran for use in NHSScotland.

8. Guidelines and Protocols

The European Society of Cardiology (ESC) published guidelines for the management of cardiomyopathies in 2023.⁴

9. Additional Information

9.1. Product availability date

18 July 2025

Table 9.1 List price of medicine under review

Medicine	Dose regimen	Cost per year (£)
vutrisiran sodium	25 mg by subcutaneous injection once every 3 months	383,449

Costs from BNF online on 03 July 2026. Costs do not take any patient access schemes into consideration.

10. Company Estimate of Eligible Population and Estimated Budget Impact

SMC is unable to publish the with-PAS budget impact due to commercial in confidence issues. A budget impact template is provided in confidence to NHS health boards to enable them to estimate the predicted budget with the PAS. This template does not incorporate any PAS discounts associated with comparator medicines or PAS associated with medicines used in a combination regimen.

*Other data were also assessed but remain confidential.**

References

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This assessment is based on data submitted by the applicant company up to and including 13 August 2026.

*[*Agreement between the Association of the British Pharmaceutical Industry \(ABPI\) and the SMC on guidelines for the release of company data into the public domain during a health technology appraisal:https://www.scottishmedicines.org.uk/about-us/policies-publications/](https://www.scottishmedicines.org.uk/about-us/policies-publications/)*

Medicine prices are those available at the time the papers were issued to SMC for consideration. SMC includes Scottish national contract prices in its decision-making. These contract prices are commercial in confidence and cannot be put in the public domain, including via the SMC Detailed Advice Document.

Patient Access Schemes (PAS): A PAS is a scheme proposed by a pharmaceutical company in order to improve the cost-effectiveness of a medicine and enable patients to receive access to cost-effective innovative medicines. The Patient Access Scheme Assessment Group (PASAG), established under the auspices of Public Services Delivery Scotland, reviews and advises NHS Scotland on the feasibility and acceptability on implementing proposed schemes. PASAG operates separately from SMC to maintain the integrity and independence of the assessment process of the SMC. When SMC accepts a medicine for use in NHS Scotland on the basis of a PAS that has been considered acceptable for implementation by PASAG, a set of guidance notes on the operation of

the scheme will be circulated to health boards' Area Drugs and Therapeutics Committees prior to publication of SMC advice.

Advice context:

No part of this advice may be used without the whole of the advice being quoted in full.

This advice represents the view of the Scottish Medicines Consortium and was arrived at after careful consideration and evaluation of the available evidence. It is provided to inform the considerations of Area Drug & Therapeutics Committees and NHS Boards in Scotland in determining medicines for local use or local formulary inclusion. This advice does not override the individual responsibility of health professionals to make decisions in the exercise of their clinical judgement in the circumstances of the individual patient, in consultation with the patient and/or guardian or carer.