NICE National Institute for Health and Care Excellence

NICE guidance

Vutrisiran for treating transthyretin amyloidosis with cardiomyopathy

Technology appraisal guidance
Published: 10 December 2025

www.nice.org.uk/guidance/ta1115

Your responsibility

The recommendations in this guidance represent the view of NICE, arrived at after careful consideration of the evidence available. When exercising their judgement, health professionals are expected to take this guidance fully into account, alongside the individual needs, preferences and values of their patients. The application of the recommendations in this guidance is at the discretion of health professionals and their individual patients and do not override the responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian.

All problems (adverse events) related to a medicine or medical device used for treatment or in a procedure should be reported to the Medicines and Healthcare products Regulatory Agency using the Yellow Card Scheme.

Commissioners and/or providers have a responsibility to provide the funding required to enable the guidance to be applied when individual health professionals and their patients wish to use it, in accordance with the NHS Constitution. They should do so in light of their duties to have due regard to the need to eliminate unlawful discrimination, to advance equality of opportunity and to reduce health inequalities.

Commissioners and providers have a responsibility to promote an environmentally sustainable health and care system and should assess and reduce the environmental impact of implementing NICE recommendations wherever possible.

Contents

1 Recommendations

1.1 Vutrisiran can be used, within its marketing authorisation, as an option to treat wild-type or hereditary transthyretin amyloidosis with cardiomyopathy in adults. Vutrisiran can only be used if the company provides it according to the commercial arrangement.

1.2 Use the least expensive option of the suitable treatments (including vutrisiran and tafamidis), having discussed the advantages and disadvantages of the available treatments with the person with the condition. Take account of administration costs, dosages, price per dose and commercial arrangements.

What this means in practice

Vutrisiran must be funded in the NHS in England for the condition and population in the recommendations, if it is considered the most suitable treatment option. Vutrisiran must be funded in England within 90 days of final publication of this guidance.

There is enough evidence to show that vutrisiran provides benefits and value for money, so it can be used routinely across the NHS in this population.

NICE has produced tools and resources to support the implementation of this guidance.

Why the committee made these recommendations

Usual treatment for transthyretin amyloidosis with cardiomyopathy is tafamidis.

The clinical trial was not designed to directly compare vutrisiran with tafamidis, and the results from indirect comparisons are uncertain. But the results from both analyses suggest that people having vutrisiran live for about as long as people having tafamidis, and that vutrisiran delays the condition getting worse at a similar rate as tafamidis.

The costs for vutrisiran are similar to or lower than for tafamidis. So, because the clinical

evidence suggests that vutrisiran is likely to work as well as tafamidis, vutrisiran can be used.

2 Information about vutrisiran

Marketing authorisation indication

2.1 Vutrisiran (Amvuttra, Alnylam Pharmaceuticals) is indicated for the 'treatment of wild-type or hereditary transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR-CM)'.

Dosage in the marketing authorisation

2.2 The dosage schedule is available in the summary of product characteristics for vutrisiran.

Price

2.3 The list price of vutrisiran is £95,862.36 per 25-mg pre-filled syringe.

2.4 The company has a commercial arrangement. This makes vutrisiran available to the NHS with a discount. The size of the discount is commercial in confidence.

Carbon Reduction Plan

2.5 For information, Alnylam did not disclose its Carbon Reduction Plan for UK carbon emissions.

3 Committee discussion

The evaluation committee considered evidence submitted by Alnylam Pharmaceuticals, a review of this submission by the external assessment group (EAG), and responses from stakeholders. See the committee papers for full details of the evidence.

The condition

Details of the condition

3.1 Transthyretin amyloidosis (ATTR) is caused by abnormal transthyretin (TTR) proteins being produced by the liver and accumulating as deposits in tissues of the body (amyloidosis). Transthyretin amyloidosis with cardiomyopathy (ATTR-CM) is a type of ATTR in which most deposits accumulate in the heart. People with ATTR-CM may also have amyloid deposits in other systems of the body. There are 2 types of ATTR-CM, both of which are more common in men than women:

- Wild-type ATTR-CM, which is more common. Symptoms usually start in people aged 70 and over.
- Hereditary ATTR-CM, which affects people born with inherited mutations in the TTR gene. Symptoms usually start in people aged 60 and over.

ATTR-CM is a progressive, life-limiting and debilitating condition. It can cause shortness of breath, palpitations, abnormal heart rhythms such as atrial fibrillation or atrial flutter, ankle swelling, fatigue and chest pain. The clinical experts explained that the rate of progression is highly variable. They said ATTR-CM progresses more quickly and is fatal without disease-modifying therapy.

Burden of disease

3.2 Patient groups explained that ATTR-CM affects every aspect of life and has

severe physical, financial and emotional effects for people with the condition. They emphasised that ATTR-CM has a major impact on the lives of carers, who are often elderly spouses or children of people with the condition. The patient expert highlighted that people often experience delayed or inaccurate diagnosis because of the rarity of the condition. They also said that lack of access to care close to home is a challenge.

Clinical management

Current treatment

3.3 NICE recommends tafamidis for treating ATTR-CM (NICE technology appraisal guidance 984). Clinical experts said that tafamidis is standard care for almost all people with ATTR-CM who are eligible for disease-modifying therapy. There are no other disease-modifying therapies available for ATTR-CM. This means that there are no treatment options for people who cannot have tafamidis or whose condition responds poorly to it. The clinical experts also noted that there had been recent improvements in other aspects of standard care for treating symptoms of the condition, including the use of sodium–glucose cotransporter-2 (SGLT2) inhibitors and improvements in imaging.

Intervention and comparators

3.4 In the final scope for this evaluation, tafamidis and established clinical management without vutrisiran were included as comparators. But the company only modelled a comparison with tafamidis. The EAG thought that a comparison with best supportive care would be relevant for people with ATTR-CM for whom tafamidis is unsuitable. It also noted that there is randomised controlled trial data available comparing vutrisiran and best supportive care from the key clinical trial. The company said that almost all people with ATTR-CM in the UK who are eligible for disease-modifying therapy have tafamidis. It also highlighted that tafamidis has only 1 contraindication and that this is rare. So, the company argued that best supportive care was not a relevant comparator. The clinical experts confirmed that almost all people eligible for disease-modifying therapy have

tafamidis in clinical practice. Patient and professional organisations suggested that it may be desirable to use tafamidis in combination with vutrisiran if both were available. But, the clinical experts explained that, if vutrisiran becomes available, it would be used alone as an alternative to tafamidis for people who prefer the method of administration or whose condition responds poorly to tafamidis. There was insufficient evidence to support their use in combination. They also said any people who cannot take tafamidis because of a comorbidity would also be ineligible for vutrisiran. The committee was satisfied that almost all people who would be eligible for vutrisiran in clinical practice currently have tafamidis. So, it concluded that tafamidis was the only relevant comparator for this evaluation and that vutrisiran would only be used as monotherapy in practice.

Clinical evidence

HELIOS-B trial

3.5 The clinical evidence for vutrisiran came from HELIOS-B, a phase 3, randomised, double-blind, placebo-controlled trial in people with ATTR-CM. People were randomly assigned to vutrisiran or placebo. People who were having tafamidis before randomisation were allowed to continue having tafamidis during the trial. The primary endpoint was a composite measure of all-cause mortality and recurrent cardiovascular events up to 36 months. Vutrisiran resulted in a significant reduction in the risk of mortality and recurrent cardiovascular events compared with placebo. In the overall population (which included people having background tafamidis at baseline and people who had not had tafamidis at baseline), the hazard ratio for the primary endpoint was 0.72 (95% confidence interval [CI] 0.56 to 0.93). In the monotherapy population (which excluded people having background tafamidis at baseline), the hazard ratio for the primary endpoint was 0.67 (95% CI 0.49 to 0.93). Some people who were not having tafamidis before the start of the trial started treatment with tafamidis during the trial. The proportion of people in the monotherapy population who started having tafamidis during the trial was similar in both the vutrisiran and placebo groups. The clinical experts stated that this was unlikely to have affected the results when considering the comparison with placebo. The committee concluded that vutrisiran was more effective for treating ATTR-CM than placebo.

Uncertainty in comparative clinical effectiveness

3.6 The company compared vutrisiran monotherapy with tafamidis monotherapy using 2 populations from HELIOS-B. The vutrisiran monotherapy group included people in the vutrisiran arm of the trial who were not having tafamidis at baseline. The tafamidis monotherapy group included people in the placebo arm who were having background tafamidis at baseline and could continue to have tafamidis during the trial. Because this comparison was not randomised, the company used inverse probability of treatment weighting to balance baseline characteristics between the 2 groups. The company presented Kaplan–Meier curves comparing all-cause mortality data for both groups. The hazard ratio for all-cause mortality using this comparison was 0.81 (95% CI 0.50 to 1.34), suggesting a possible improvement with vutrisiran but lacking statistical significance. The company said this within-trial comparison provided the most robust assessment of relative efficacy. It argued that indirect treatment comparisons would not be appropriate. This was because the populations in HELIOS-B and the pivotal trial for tafamidis (ATTR-ACT) were not comparable, because the participants in ATTR-ACT had more advanced disease than in HELIOS-B. The company highlighted that there was potential for bias in favour of tafamidis in the within-trial comparison, because people in the tafamidis monotherapy group had been having tafamidis for a median of 11.3 months at baseline. This meant that people in the tafamidis monotherapy group may have had a survival benefit before the start of the trial. The EAG said that this was plausible but difficult to assess because the company had not provided any evidence to explore the impact of different treatment start times. The EAG also noted several uncertainties associated with the company's comparison, including that:

- the within-trial comparison was non-randomised and HELIOS-B was not designed to compare vutrisiran with tafamidis
- some people in the vutrisiran monotherapy group started treatment with tafamidis after the start of the trial, which complicated the analysis
- the results for all-cause mortality and other endpoints were not statistically significant
- the Kaplan–Meier curves for all-cause mortality did not reflect the company's conclusions about the effectiveness of vutrisiran compared with tafamidis.

Although the EAG agreed that HELIOS-B and ATTR-ACT had different populations, it disagreed that this made indirect treatment comparisons inappropriate. This was because population adjustment methods, such as matching-adjusted indirect comparisons (MAICs), are designed to adjust for effect modifiers and prognostic factors. The company provided unanchored and anchored MAICs (the results of which were similar to the within-trial comparison results but are confidential and cannot be reported here). The EAG did its own network meta-analysis (NMA) to compare HELIOS-B and ATTR-ACT. The hazard ratio for all-cause mortality in the EAG's NMA was similar to that of the company's within-trial comparison and was not statistically significant: 0.89 (95% CI 0.53 to 1.43). The EAG also did an exploratory NMA comparing HELIOS-B with a post-2019 cohort of the Transthyretin Amyloidosis Outcomes Survey (THAOS) who had tafamidis monotherapy. THAOS, a source of real-world evidence, collected ATTR natural history data from over 6,000 people from 19 countries (excluding the UK). The hazard ratio for the NMA comparing HELIOS-B with THAOS was 1.17 (95% CI 0.73 to 1.90). The EAG said there was no conclusive evidence that vutrisiran was more effective than tafamidis because no analyses were statistically significant. Although some results suggested a potential clinical benefit for vutrisiran, others suggested a potential clinical benefit for tafamidis. The clinical experts said that it was difficult to compare the 2 treatments given the trial data and there was no strong evidence of a clinical benefit for vutrisiran compared with tafamidis.

The committee considered the analyses presented and noted that none of the hazard ratios were used directly in the model. It noted the high uncertainty associated with the comparison of vutrisiran and tafamidis and the lack of statistical significance for any outcomes presented. It also noted the clinical expert view that the presented evidence did not support a difference in clinical benefit between treatments. So, the committee preferred to assume that vutrisiran and tafamidis had equal clinical effectiveness.

Adverse events

3.7 The incidence of some serious adverse events was higher in the tafamidis group

than the vutrisiran group. But the EAG highlighted that the incidence of other serious adverse events was higher in the vutrisiran group than the tafamidis group and felt that this was not reflected in the modelling. Also, the incidence of adverse events was not adjusted for differences in baseline characteristics, which added to the uncertainty. The committee concluded that it was reasonable to assume similar adverse event profiles between vutrisiran and tafamidis.

Economic model

Company's modelling approach

3.8 The company modelled the costs and effects of vutrisiran using a cohort-level Markov state-transition model. The model included 4 'alive' health states based on the 4 New York Heart Association (NYHA) classes (1, 2, 3 and 4). People could stay in their NYHA class, move to a less severe NYHA class, move to a more severe NYHA class, or transition to the 'death' health state. The contribution of each NYHA health state to the death health state was proportional to the number of people in the health state and the relative mortality hazard in that NYHA class. The risk of transient cardiovascular event occurrence depended on NYHA class. Treatment-independent health state utilities included in the model were measured in HELIOS-B using the EQ-5D-5L and mapped to the EQ-5D-5L. The model cycle length was 3 months and a lifetime horizon was used.

The EAG noted that the model structure was broadly consistent with previous models considered in NICE evaluations for ATTR-CM. But it was concerned that the model structure did not appropriately link survival and NYHA health states or transient cardiovascular events. It said that the lack of structural link between survival and disease progression led to implausible survival extrapolations that needed logical constraints. The company said that a competing-risks or multi-state survival analysis could formally link overall survival to NYHA class transitions, but that this was not advisable. This was because a small number of people were in some NYHA classes at baseline and there was a low number of mortality events in HELIOS-B. So it thought that there was insufficient data to derive robust NYHA class-specific survival estimates. The committee concluded that the company's model structure was acceptable given the available data. But

it also concluded that the lack of structural link between survival and disease progression led to a high level of uncertainty in the economic modelling.

Modelling overall survival

3.9 The company modelled overall survival (OS) by fitting parametric curves independently to the inverse probability of treatment weighting-adjusted OS data for the vutrisiran monotherapy and tafamidis monotherapy groups (see section 3.6). For vutrisiran it preferred to use the log-logistic distribution and for tafamidis it preferred the log-normal distribution. Both extrapolations were capped by age- and sex-matched general population mortality. The company noted that the goodness-of-fit statistics were similar across the parametric distributions. It also said that its chosen extrapolations aligned with the observed hazards in HELIOS-B and that clinical experts deemed them plausible in a structured elicitation exercise.

The EAG thought that the company's approach to modelling OS was associated with high uncertainty. It said that the company's preferred extrapolations likely overestimated the relative treatment effect. The EAG explained that the lack of structural link between survival and disease progression (see section 3.8) meant that the extrapolations had to be capped by the general population mortality curve. It noted that the log-logistic and log-normal curves assumed decreasing hazards over time, which it considered implausible in the long term. For vutrisiran, the EAG preferred to use the exponential distribution because it was the only parametric function that did not assume decreasing hazards in the long term. Because it preferred to assume there was no treatment effect for vutrisiran compared with tafamidis, the EAG preferred to use the same OS curve for both interventions. The committee considered the company and EAG's approaches. It noted that the OS data was immature because of the low mortality in HELIOS-B. This meant that the extrapolations beyond the trial period were highly uncertain. The committee recalled its preference to assume that vutrisiran and tafamidis had equal clinical effectiveness. So, it preferred the EAG's approach for modelling OS. That is, to fit the exponential distribution to the vutrisiran OS data and to use the same curve for tafamidis.

NYHA health state-transition probabilities

3.10 The company derived transition matrices separately for vutrisiran and tafamidis from the double-blind period of HELIOS-B, in which NYHA class was collected at 6-month intervals. For the first 30 months of the time horizon, 6-month transition matrices were estimated from the observed data and converted to 3-month transition matrices to align with the cycle length. Beyond 30 months, the average of the last 2 observed 6-month transition matrices was converted to a 3-month transition matrix and carried forward for the remainder of the time horizon. The company used this approach because it thought that the transition matrices for the last observed period would have been implausible for the long term. The EAG was concerned that the transition matrices used in the model may not accurately reflect disease progression over the time horizon. It said that some of the transition probabilities lacked face validity and were likely driven by the distribution of disease severity at HELIOS-B baseline. The clinical experts explained that few people reach NYHA class 4 and that most people die in NYHA 3. Because of this, the clinical experts said the differences in health state distribution over the modelled time horizon between the vutrisiran and tafamidis arms did not align with what they see in clinical practice. The committee noted that the large changes in health state membership at some time points also added to the lack of plausibility of the transition matrices. It noted that the transition matrices were informed by the inverse probability of treatment weighting analysis and the weighting used may also have contributed to the implausibility of some of the transition probabilities. The committee recalled its preference to assume that vutrisiran and tafamidis had equal effectiveness. So, it preferred to assume that the transition matrices were the same for vutrisiran and tafamidis.

Vutrisiran treatment effect waning

3.11 The company included treatment effect waning from 12 months after vutrisiran was stopped. This was only modelled for vutrisiran because the company believed that its mechanism of action as a TTR silencer meant that serum TTR levels would remain low for a sustained period after treatment is stopped. The company justified not modelling treatment effect waning for tafamidis because it is not expected to maintain active pharmacodynamic levels without sustained

daily administration. Clinical experts consulted by the company said that around an 80% reduction in serum TTR compared with baseline indicated a remaining treatment effect for people with ATTR-CM. The company provided data on the mean change in serum TTR from baseline after the final dose of vutrisiran in the double-blind period of HELIOS-B (this data is confidential and cannot be reported here).

The EAG said that a gradual treatment effect loss was plausible but the company may not have modelled this appropriately. It said that there was a lack of empirical evidence that an 80% reduction in serum TTR compared with baseline indicated a remaining treatment effect. It also noted that the company's modelling of treatment effect waning implied a sudden treatment effect loss 12 months after stopping instead of a gradual treatment effect waning period, in line with the gradual decrease in TTR serum levels shown in HELIOS-B. The clinical experts explained that people taking vutrisiran are likely to experience a clinical benefit after stopping treatment because it takes around 18 months for amyloid production to return to baseline levels after stopping vutrisiran. The clinical experts also said that an 80% reduction in serum TTR indicates clinical benefit, but acknowledged there was no empirical evidence for this assumption. They also noted that the treatment discontinuation rate for vutrisiran was low and usually linked to toxicity or was because disease progressed beyond a point where continued treatment would improve quality of life. The committee was satisfied that a treatment waning effect was plausible for vutrisiran. But it noted that treatment discontinuation was rare and it was uncertain if the company's modelling was appropriate. The committee also recalled its preference to assume that vutrisiran and tafamidis had equal clinical effectiveness. So, it preferred to assume no treatment effect after stopping either vutrisiran or tafamidis, but would consider the potential treatment waning effect of vutrisiran as an uncaptured benefit.

Carer disutilities

3.12 The company included carer disutilities in its analysis. It explained that ATTR-CM has a substantial impact on the lives of carers, who are often elderly spouses or children of people with the condition. The company noted that carer disutilities had been accepted in previous NICE evaluations for hereditary ATTR with

polyneuropathy (hATTR-PN), including in NICE's highly specialised technology guidance on inotersen for treating hereditary transthyretin amyloidosis (HST9) and patisiran for treating hereditary transthyretin amyloidosis (HST10). It thought that the carer burden in ATTR-CM was similar to, if not higher than, that in hATTR-PN. Because it could not identify any suitable evidence for the carer burden in ATTR-CM, the company sourced carer disutilities from a study measuring EQ-5D-5L in 36 carers of people with hereditary ATTR reported by familial amyloid polyneuropathy (FAP) stage of the people they were caring for. This study was used in HST9, in which health states were based on FAP stage. The company used the FAP stages as a proxy for NYHA class in this evaluation.

The EAG thought that it was uncertain whether carer disutilities should be included in this evaluation. It was also uncertain whether the carer burden in ATTR-CM was comparable to that in hATTR-PN and whether FAP staging could be reliably mapped to NYHA class. The patient expert explained that ATTR-CM has a substantial impact on carers, including financial, emotional and psychological impacts. They said that carers often experience chronic fatigue and isolation and that the carer burden in ATTR-CM is comparable to that in hATTR-PN. The clinical experts said that it was difficult to compare FAP staging with the NYHA classification because cardiomyopathy and polyneuropathy have different symptoms. The committee felt that the condition was associated with a substantial effect on carer health-related quality of life and that it was appropriate to consider carer burden in this evaluation. But it was uncertain whether the number of carers and carer disutilities modelled by the company reflected the carer burden in ATTR-CM. The committee felt that the company's modelling of carer disutilities was not fully evidenced or justified. It recalled its preference to assume that vutrisiran and tafamidis had equal clinical effectiveness. Because this meant assuming no treatment effect, including carer disutilities had no impact on the cost-effectiveness estimates. So, the committee concluded that it was not applicable to include the company's carer disutilities in the model.

Costs

Vutrisiran administration cost

3.13 The company assumed that vutrisiran treatment would start in hospital and subsequent administration would be done at home with the availability of company-funded homecare. It said this aligned with the latest summary of product characteristics for vutrisiran. The company also thought that this was a conservative assumption because the National Amyloidosis Centre intends for all people having vutrisiran to start treatment at home in the future. The EAG assumed that treatment initiation and continuation would be done by a healthcare professional throughout time on treatment. It said this was because it could not verify the information provided by the company. The clinical experts confirmed that continued treatment is done at home with the availability of company-funded homecare and that treatment may also be started at home in the near future. The committee was satisfied that treatment is currently started in hospital and that subsequent administration takes place at home.

Tafamidis acquisition cost

3.14 The company excluded within-cycle correction for both the tafamidis and vutrisiran acquisition costs. The EAG agreed with excluding within-cycle correction for the vutrisiran acquisition cost because it is administered every 3 months, which aligns with the model cycle length. But the EAG included within-cycle correction for the tafamidis acquisition cost. The company explained that tafamidis is supplied every 3 months, but the EAG said it could not verify this information. The clinical experts confirmed that tafamidis is supplied every 3 months. So, the committee agreed that within-cycle correction should be excluded for both vutrisiran and tafamidis acquisition costs.

Cost-effectiveness estimates

3.15 The committee noted the high level of uncertainty, specifically:

- There was a high level of uncertainty in the clinical-effectiveness evidence comparing vutrisiran with tafamidis (section 3.6).
- The model structure did not appropriately link survival and disease progression, which led to uncertainties in the modelling (section 3.8).
- The company's preferred parametric extrapolations were highly uncertain (section 3.9).
- The transition probabilities lacked face validity (section 3.10).
- The company's modelling approach for treatment effect waning was uncertain (section 3.11).
- The company's carer disutilities were not fully evidenced or justified (section 3.12).

Despite the uncertainties, the committee concluded that vutrisiran is likely to work as well as tafamidis. So, it preferred to assume that vutrisiran and tafamidis had equal clinical effectiveness. The committee concluded that, to be recommended as a treatment option, vutrisiran had to have similar or lower costs to tafamidis.

A cost-minimisation analysis suggested that the costs for vutrisiran are similar to or lower than for tafamidis. So, the committee concluded that vutrisiran is likely to be a cost-effective use of NHS resources.

Other factors

Equality

3.16 The committee considered the following potential equalities issues:

- ATTR-CM primarily affects older people.
- Hereditary ATTR-CM disproportionately affects people with certain genetic variants (such as Val122Ile), which are more prevalent in African, Caribbean

and Hispanic populations.

Age and race are protected characteristics under the Equality Act 2010. But because its recommendations do not restrict access to treatment for some people over others, the committee agreed that the recommendations would not have a different effect on people protected by equality legislation than on the wider population. The equalities issues raised related to prevalence of the condition could not be addressed by this evaluation.

Uncaptured benefits

3.17 The committee considered whether there were any uncaptured benefits of vutrisiran. It identified the following additional benefits of vutrisiran not captured in the economic modelling when using a cost-minimisation analysis:

- A treatment effect waning period after discontinuation was thought plausible for vutrisiran (see section 3.11). But, the committee agreed that treatment discontinuation is likely to affect a small number of people and noted that it had a small impact on the cost-effectiveness estimates.
- The committee felt that the condition was associated with a substantial effect on carer health-related quality of life and was satisfied that it was appropriate to consider carer burden in this evaluation (see section 3.12). But, by assuming there is no treatment effect benefit with vutrisiran compared with tafamidis, it was also assumed that the impact on carer quality of life would be the same for vutrisiran and tafamidis.
- Vutrisiran is administered less frequently than tafamidis, which may be convenient for some people with ATTR-CM.

Conclusion

Recommendation

3.18 The committee noted the important uncertainties in the clinical-effectiveness

evidence and economic modelling. But it concluded that vutrisiran is likely to have similar clinical effectiveness to tafamidis and that the uncertainties could not be addressed with the available data. The costs for vutrisiran are also similar to or lower than for tafamidis. So, vutrisiran can be used for treating ATTR-CM.

4 Implementation

4.1 Section 7 of the National Institute for Health and Care Excellence (Constitution and Functions) and the Health and Social Care Information Centre (Functions) Regulations 2013 requires integrated care boards, NHS England and, with respect to their public health functions, local authorities to comply with the recommendations in this evaluation within 90 days of its date of publication.

4.2 Section 4f of The Innovative Medicines Fund Principles states that a discretionary source of early funding (from the overall Innovative Medicines Fund budget) is available for certain medicines recommended by NICE. In this instance, interim funding has been agreed for vutrisiran. Interim funding will end 90 days after positive final guidance is published (or 30 days in the case of drugs with an Early Access to Medicines Scheme designation or cost comparison evaluation), at which point funding will switch to routine commissioning budgets.

4.3 The Welsh ministers have issued directions to the NHS in Wales on implementing NICE technology appraisal guidance. When a NICE technology appraisal guidance recommends the use of a drug or treatment, or other technology, the NHS in Wales must usually provide funding and resources for it within 60 days of the first publication of the final draft guidance.

4.4 When NICE recommends a treatment 'as an option', the NHS must make sure it is available within the period set out in the paragraphs above. This means that, if a patient has transthyretin amyloidosis with cardiomyopathy and the healthcare professional responsible for their care thinks that vutrisiran is the right treatment, it should be available for use, in line with NICE's recommendations.

5 Evaluation committee members and NICE project team

Evaluation committee members

The 4 technology appraisal committees are standing advisory committees of NICE. This topic was considered by committee C.

Committee members are asked to declare any interests in the technology being evaluated. If it is considered there is a conflict of interest, the member is excluded from participating further in that evaluation.

The minutes of each evaluation committee meeting, which include the names of the members who attended and their declarations of interests, are posted on the NICE website.

Chair

James Fotheringham
Vice chair, technology appraisal committee A

NICE project team

Each evaluation is assigned to a team consisting of 1 or more health technology analysts (who act as technical leads for the evaluation), a technical adviser, a project team and an associate director.

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Project team

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ISBN: 978-1-4731-7461-0