IMPACT OF THE SOCIETAL PERSPECTIVE ON HTA DECISION-MAKING IN RARE DISEASES

HTA195

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Introduction and aims

- While many European Health Technology Assessment (HTA) guidelines state that the societal perspective should be considered in their assessments, it's inclusion in economic evaluations and subsequent influence on decision-making is unknown.
- This is particularly impactful for rare diseases, where healthcare payer perspectives are prioritised due to limited or highly uncertain data on the cost of treatments, or the disease itself, to society. For example, in the United Kingdom (UK), the National Institute for Health and Care Excellence (NICE) states that wider costs are only incorporated "in exceptional circumstances for medicines."[1]
- The true, broader value of a new treatment for a rare disease is therefore potentially underrecognised.
- Recent updates to Dutch guidelines recommend including spillover health effects (impact on families and caregivers) and informal care costs (education and justice [equitable social impact]) in their economic reference case.[2]

This research aims to understand how frequently a societal perspective is considered among European HTA bodies and the impact it has on decision making.

Methods

- A targeted literature review (TLR) was conducted to identify rare disease appraisals published from 1st January 2022 to 20th May 2025, among HTA bodies that consider cost-effectiveness, including: the UK (NICE), the Netherlands (Zorginstituut Nederland [ZIN]), Finland (Finnish Medicines Agency [FIMEA]) and the Nordics (Joint Nordic HTA-Bodies [JNHB]).
- HTA agency websites were searched using key terms such as 'rare' (NICE) or cross-referenced against the MHRA orphan drug list (ZIN, FIMEA, and JNHB).[3]
- Outcomes pertaining to the societal impact of treatments were identified through keyword searches of patient education, patient or caregiver employment, and patient or caregiver participation in daily activities.
- Appraisals were searched for information related to societal perspective scenarios in the economic evaluation.

Results

Reporting of overall societal impact:

- Of the 412 total appraisals identified at initial screening, 110 rare disease appraisals were included (NICE, 61; ZIN, 37; FIMEA, 8; JNHB, 4).
- Societal impact of the disease or new treatment was reported in 66.4% (73/110) of rare disease appraisals, most commonly in ZIN appraisals (**Figure 1**).

The impact of rare diseases on participation on education/employment:

At least one rare disease appraisal submitted to each HTA body considered the impact of the disease on education and/or employment of the patient and caregiver (**Figure 2**).

Figure 1: Proportion of rare indication appraisals that reported societal impact on patients or caregivers

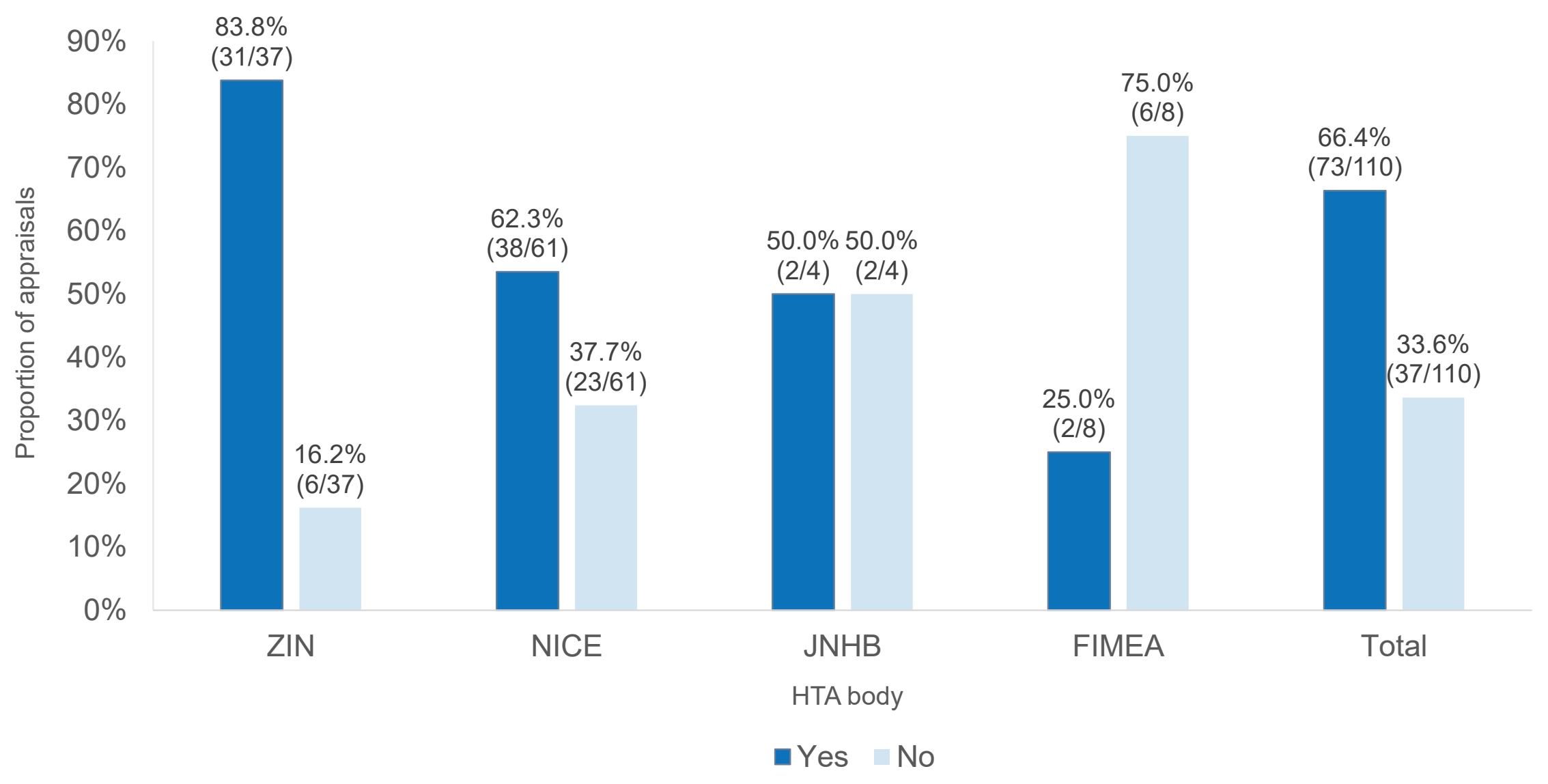
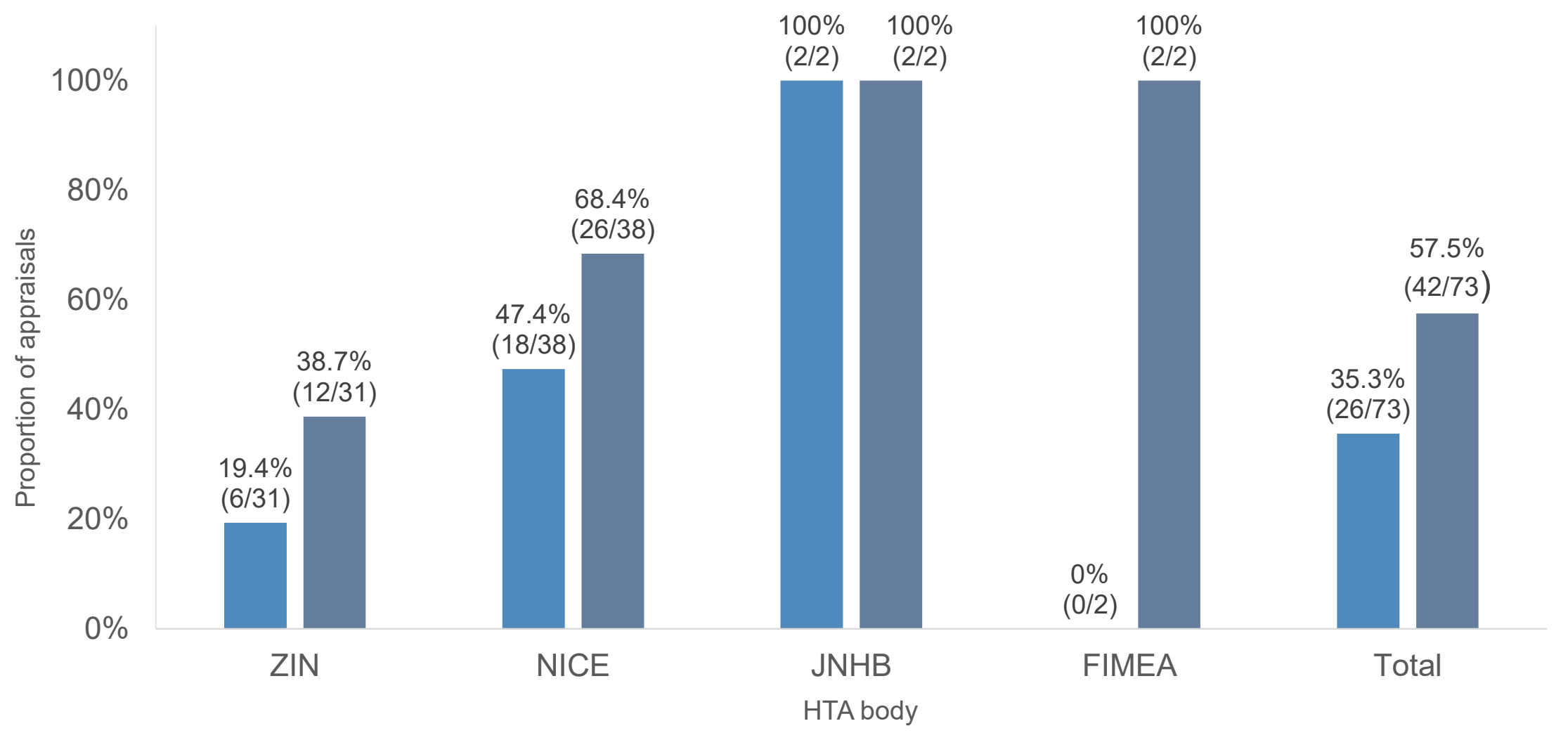


Figure 2: Proportion of rare indication appraisals that reported impact on education and/or employment



FIMEA: Finnish Medicines Agency; HTA: Health technology assessment; JNHB: Joint Nordic HTA-Bodies; NICE: National Institute of Health and Care Excellence; ZIN: Zorginstituut Nederland.

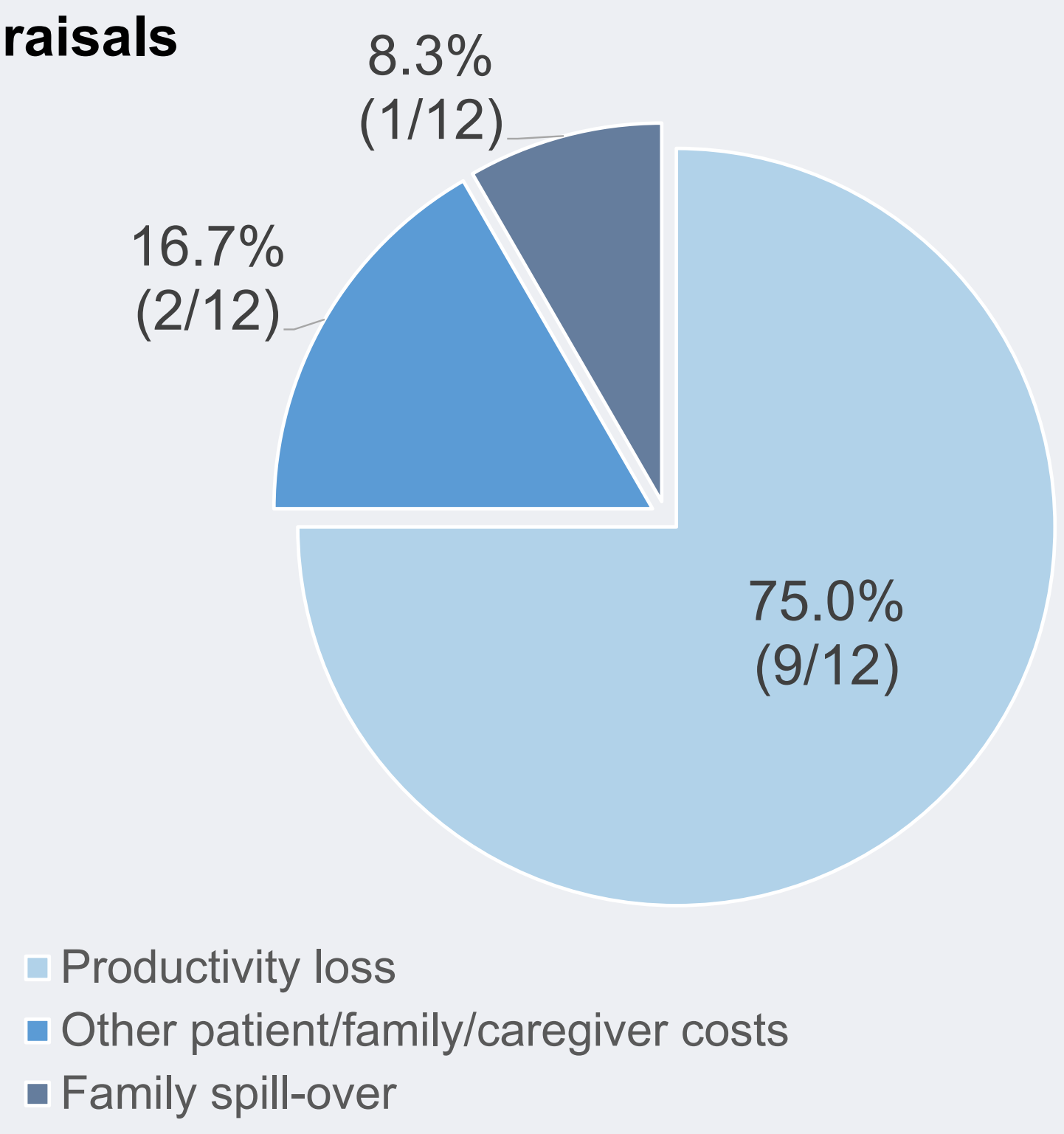
Results (continued)

Influence of societal perspective on the cost-effectiveness analysis:

Of the 73 appraisals that reported societal impact, 50.7% (37/73) incorporated societal costs in their economic evaluations:

- For ZIN, 74.2% (23/38) of appraisals utilised a societal perspective as the base case; none as a scenario.
- For NICE, 2.6% (1/38) appraisal utilised a societal perspective as the base case, and 29.0% (11/38) included a societal perspective as a scenario.
- For JNHB and FIMEA, 50% (1/2) of appraisals from each HTA body utilised a societal perspective as the base case; none as a scenario.
- For NICE, societal costs considered in economic evaluations included productivity loss, other costs (associated with travel to appointments, out-of-pocket expenses associated with informal care), or family spill-over (bereavement) (**Figure 3**).

Figure 3: Societal costs considered in economic evaluations for NICE appraisals



Two appraisals utilised both productivity loss and patient/family/caregiver costs, and two appraisals did not specify category of indirect costs.
NICE: National Institute of Health and Care Excellence.

For NICE appraisals, 18.4% (7/38) reported incremental cost-effectiveness ratios (ICERs) for both the healthcare payer and societal perspective. The ICERs when considering a societal perspective were generally lower than the base case, by up to 55% (**Table 1**).

In HST30 specifically, the societal perspective resulted in the committee allowing more flexibility in determining the ICER threshold.[4]

Table 1: The impact of a societal perspective on the ICER among NICE appraisals

Appraisal number	ICER, £/QALY Healthcare payer's perspective base case	Societal perspective scenario	Difference	Indirect cost considered
TA1011*	42,997	35,597	-17.2%	N/A**
	33,490	28,041	-16.3%	
	45,676	35,367	-22.6%	
HST22	145,514	145,897	+0.3%	Productivity loss
HST23*	80,093	74,689	-6.8%	Productivity loss
	98,512	98,303	-0.2%	
HST29	101,073	153,717	+52.1%†	Productivity loss
		88,224	-12.7%	Additional caregivers
HST30	239,608	230,490	-3.8%	Family health spill-over
		149,400	-37.7%	Productivity loss
		143,400	-40.2%	Both
TA955	26,776	12,158	-54.5%	Productivity loss
TA937	18,643	14,453	-22.5%	N/A**

*Appraisals reported ICERs for multiple cohorts separately.
**Specific indirect costs in the societal perspective were not reported.
†The NICE committee recognised the complexity and counterintuitive results from the productivity loss scenario in HST29.
HST: Highly specialised technology; ICER: Incremental cost-effectiveness ratio; QALY: Quality-adjusted life years; TA: Technology appraisal.

In addition to Table 1, in HST33, while relevant ICER data were not reported, the NICE committee "accepted a higher level of uncertainty in clinical evidence and modelling than would normally be accepted" with a societal perspective.[5]

Exploratory analysis: Paediatric NICE appraisals (non-rare disease indications)

The original research question was expanded to include a broader scope and understand the impact of the societal perspective on NICE appraisals for paediatric indications.

- Of the 16 additional NICE appraisals that were extracted, 31.3% (5/16) presented a societal perspective as a scenario analysis in the model.
- Two appraisals (TA1003 and TA814) reported a reduction in ICER when the societal perspective was applied, although values were redacted.
- TA1034, which evaluated anhydrous sodium thiosulfate for preventing hearing loss caused by cisplatin chemotherapy in a paediatric population, reported a 65% improvement in the ICER with a societal perspective vs the base case.[6] The societal costs considered in TA1034 were related to education, productivity loss for parents, and productivity loss for patients when they reach working age. The improvement in ICER was primarily driven by education costs.

Discussion

- Societal impact was reported most frequently (83.7%) in appraisals by ZIN, in line with recently published Dutch HTA guidelines that put explicit emphasis on considering the societal perspective in decision-making.
- Of the NICE appraisals in which a societal scenario was considered in the economic model, the ICER improved by up to 54% vs the base case.
- Patient and clinical experts, and the HTA committee itself, commented on the societal impact of the disease in all HTA bodies included in this TLR, highlighting the influence societal perspectives may have on decision-making.
- The societal perspective directly influenced decision-making in some NICE appraisals through more favourable ICERs, greater acceptance of uncertainty, or an adjusted willingness-to-pay threshold.

Conclusions

- Rare and paediatric diseases pose a significant burden to the patient, their support network, and wider society. This burden is often not captured in HTA appraisals when the reference case is from a healthcare payer's perspective.
- Apart from ZIN, most major cost-effectiveness HTA bodies across Europe do not consider the societal perspective in the reference case. By not including societal perspective in rare and non-rare disease appraisals, the true value of treatments may not be fully captured through the healthcare payer perspective alone, potentially leading to suboptimal decision-making that impacts patient access to important treatments.
- Manufacturers preparing for rare disease HTA should aim to capture robust data demonstrating the wider societal impact and include the societal perspective in their submissions.
- HTA bodies should recognise the impact of the societal perspective and follow in ZIN's footsteps to consider societal costs in the economic model reference case.

Limitations

The TLR was limited to the information reported in the HTA appraisal documents. Additionally, the scope of the research was limited to a small number of geographies in which cost-effectiveness is influential to decision-making.

References

1. 1. NICE health technology evaluations: the manual. https://www.nice.org.uk/process/pmg36. Accessed 19/08/2025.
2. 2. H. Amarens Geuzinge, et al. The New Dutch Guideline for Economic Evaluations in Healthcare: Taking the Societal Perspective to the Next Level. Value in Health: 28 (6). 2025. https://doi.org/10.1016/j.jval.2025.03.002.
3. 3. MHRA Orphan Register. https://www.gov.uk/government/publications/orphan-registered-medicinal-products/orphan-register. Accessed 20/05/2025.
4. 4. NICE. HST30: Sebelipase alfa for treating Wolman disease. https://www.nice.org.uk/guidance/hst30. Accessed 20/05/2025.
5. 5. NICE. HST33: Leniolisib for treating activated phosphoinositide 3-kinase delta syndrome in people 12 years and over. https://www.nice.org.uk/guidance/hst33. Accessed 17/07/2025.
6. 6. NICE. TA1034: Anhydrous sodium thiosulfate for preventing hearing loss caused by cisplatin chemotherapy in people 1 month to 17 years with localised solid tumours. https://www.nice.org.uk/guidance/ta1034/documents/html-content-10. Accessed 20/05/2025.

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