NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Sodium thiosulfate for preventing ototoxicity in people aged 1 month to 17 years with localised cancer having cisplatin chemotherapy

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of sodium thiosulfate within its marketing authorisation for preventing ototoxicity in people aged 1 month to 17 years old with localised cancer having cisplatin chemotherapy.

Background

Cisplatin is a chemotherapy that is widely used to treat a variety of cancers in children and young people. However, after it enters the cochlea (inner ear) it can cause damage, known as ototoxicity or 'ear poisoning'. Ototoxicity can impair the function of the inner ear related to balance (causing dizziness or vertigo). It can also affect hearing, such as hearing sounds in the absence of external noises (tinnitus) and hearing loss. While these impairments can sometimes be temporary, it can also cause irreversible damage resulting in, for example, permanent hearing loss. The onset of hearing loss can occur immediately or may occur progressively years after cisplatin treatment. A person's age, genetics, and cisplatin dosage can affect whether hearing loss occurs. Hearing loss can delay speech and language development in children and can have a significant impact on school performance and psychosocial functioning.

Cancer in people aged under 15 is rare compared with the adult population, accounting for less than 1% of all cancers.[1] In 2016-8 there were on average 1838 new cases of cancer in people aged under 15 per year in the UK.[1] All people who are under 18 and receiving cisplatin are at risk of hearing loss.[2] In 2022-23, there were 283 new cases of ototoxic hearing loss diagnosed in people aged under 18 in England.[3]

There are currently no treatment options available to prevent ototoxicity in people aged 1 month to 17 years with localised, solid cancer tumours having cisplatin chemotherapy.

The technology

Sodium thiosulfate (Pedmarqsi, Fennec Pharmaceuticals) has a marketing authorisation for the prevention of ototoxicity induced by cisplatin chemotherapy in patients 1 month to less than 18 years of age with localised, non-metastatic, solid tumours.

Intervention(s)	Sodium thiosulfate
Population(s)	People aged 1 month to 17 years with localised, non-metastatic tumours having cisplatin chemotherapy

Comparators	Established clinical management without sodium thiosulfate
Outcomes	The outcome measures to be considered include: • frequency of hearing loss • audiological outcomes (e.g. sound perception, speech recognition and sound localisation) • language and communication outcomes (e.g. intelligibility, sentence comprehension) • psychosocial development/adjustment • adverse effects of treatment including impact on response to cisplatin and survival • health-related quality of life.
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	**Related cancer service guidelines:** Improving outcomes in children and young people with cancer (2005) NICE guideline CSG7. **Related NICE guidelines:** Tinnitus: assessment and management (2020) NICE guideline NG155. **Related quality standards:** Cancer services for children and young people (2014) NICE quality standard 55
Related National Policy	The NHS Long Term Plan (2019) NHS Long Term Plan NHS England (2018) NHS manual for prescribed specialist services (2018/2019) Chapter 106: Specialist cancer services for children and young people

	NHS England (2013) 2013/14 Standard Contract for Paediatric Oncology NHS England (2013) 2013/14 NHS Standard Contract for Cancer: Teenagers and Young Adults NHS England B02: Chemotherapy. Clinical Reference Group. [Accessed November 2023] NHS England B05. Children and Young Adult Cancer Services. Clinical Reference Group. [Accessed November 2023] Department of Health and Social Care, NHS Outcomes Framework 2016-2017: Domains 2 - 5. https://www.gov.uk/government/publications/nhs-outcomes-framework-2016-to-2017

Questions for consultation

Is the population defined appropriately in the scope?

How many people aged between 1 month and 17 years are newly diagnosed with cancer in England and Wales each year?

Among people aged between 1 month and 17 years living with cancer in England and Wales, how many of them have localised, non-metastatic cancer?

What proportion of people aged 1 month to 17 years with localised, non-metastatic cancer are having cisplatin in England and Wales? Among those having cisplatin, how many of them experience ototoxicity? And how many experience hearing loss? What are the other potential factors that may be associated with ototoxicity and/or hearing loss in children and young people (aged 1 month to 17 years) with localised, non-metastatic cancer and having cisplatin?

For people aged 1 month to 17 years with localised, non-metastatic cancer having cisplatin, what proportion would be eligible for treatment with sodium thiosulfate to prevent ototoxicity? How would eligibility for sodium thiosulfate be determined in clinical practice?

Is the comparator defined appropriately in the scope? Have all relevant comparators been included in the scope?

Where do you consider sodium thiosulfate will fit into the existing care pathway for preventing ototoxicity in people aged 1 month to 17 years with localised, non-metastatic cancer having cisplatin chemotherapy?

Is sodium thiosulfate currently used in the NHS for treating other conditions? If so, which conditions?

Are there any other treatments or services currently used in clinical practice to prevent ototoxicity or support people with hearing loss in people aged 1 month to 17 years with localised, non-metastatic cancer and having cisplatin chemotherapy? if so, what are the treatments or services in place? Are cisplatin dose reductions used if symptoms of toxicity become evident?

Have all relevant outcomes been included in the scope?

Do you consider that the use of sodium thiosulfate can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation? If so, what these would be?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

Would sodium thiosulfate be a candidate for managed access?

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which sodium thiosulfate is licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

References

1. Cancer Research UK. Childhood cancer key statistics. Accessed November 2023
2. Brock PR, Knight KR, Freyer DR, et al. (2012) Platinum-induced ototoxicity in children: a consensus review on mechanisms, predisposition, and protection including a new International Society of Pediatric Oncology Boston Ototoxicity Scale. Journal of Clinical Oncology. 30(19): 2408-17.
3. NHS England (2023) Hospital Admitted Patient Care Activity, 2022-2023.