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Health Technology Evaluation

Anhydrous sodium thiosulfate (Pedmarqsi) for preventing ototoxicity caused by cisplatin chemotherapy in people aged 1 month to 17 years with localised solid tumours**Final scope****Remit/evaluation objective**

To appraise the clinical and cost effectiveness of anhydrous sodium thiosulfate (Pedmarqsi) within its marketing authorisation for preventing ototoxicity induced by cisplatin chemotherapy in patients 1 month to less than 18 years of age with localised, non-metastatic, solid tumours.

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 some impairments can sometimes be temporary, it can also cause irreversible damage resulting in 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

Anhydrous 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)	Anhydrous sodium thiosulfate (Pedmarqsi)
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Draft scope for the evaluation of anhydrous sodium thiosulfate (Pedmarqsi) for preventing ototoxicity caused by cisplatin chemotherapy in people aged 1 month to 17 years with localised solid tumours

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Population(s)	People aged 1 month to less than 18 years of age with localised, non-metastatic, solid tumours having cisplatin chemotherapy
Comparators	Established clinical management without anhydrous sodium thiosulfate (Pedmarqsi)
Outcomes	<p>The outcome measures to be considered include:</p> <ul style="list-style-type: none"> • frequency and severity 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	<p>The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year.</p> <p>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.</p> <p>Costs will be considered from an NHS and Personal Social Services perspective.</p>
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	<p>Related cancer service guidelines: Improving outcomes in children and young people with cancer (2005) NICE guideline CSG7.</p> <p>Related NICE guidelines: Tinnitus: assessment and management (2020) NICE guideline NG155.</p> <p>Related quality standards: Cancer services for children and young people (2014) NICE quality standard 55</p>

Related National Policy	<p>The NHS Long Term Plan (2019) NHS Long Term Plan</p> <p>NHS England (2018) NHS manual for prescribed specialist services (2018/2019) Chapter 106: Specialist cancer services for children and young people</p> <p>NHS England (2013) 2013/14 Standard Contract for Paediatric Oncology</p> <p>NHS England (2013) 2013/14 NHS Standard Contract for Cancer: Teenagers and Young Adults</p> <p>NHS England B02: Chemotherapy. Clinical Reference Group. [Accessed November 2023]</p> <p>NHS England B05. Children and Young Adult Cancer Services. Clinical Reference Group. [Accessed November 2023]</p> <p>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</p>
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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](#).