NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Pitolisant for treating narcolepsy in children and young people 6 to 17 years ID6353

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of pitolisant within its marketing authorisation for treating narcolepsy in children and young people 6 to 17 years.

Background

Narcolepsy is a chronic neurological condition affecting the brain's ability to regulate normal sleep/wake cycles. It is often caused by a lack of the brain chemical hypocretin (also known as orexin), but this does not explain all cases and the exact cause of the condition is often unclear.[1,2] Symptoms of narcolepsy can include excessive daytime sleepiness, sleep attacks (falling asleep suddenly and without warning), cataplexy (temporary loss of muscle control resulting in weakness and possible collapse), sleep paralysis, excessive dreaming, waking in the night and disturbed nocturnal sleep.[1,2] Narcolepsy can have a significant impact on daily life leading to those with the condition to become emotionally withdrawn and socially isolated.[2]

Approximately 30,000 people (1 in 2,500) have narcolepsy in the UK, although it is believed that the majority have not been diagnosed.[3] Initial symptoms occur before the age of 18 in over 50% of cases. The prevalence of narcolepsy in childhood is not known but can be estimated from adult studies to be greater than 20 to 60 per 100,000 (0.02% to 0.06%) in Western countries.[4]

Initial treatment for narcolepsy in children and young people consists of environmental and behavioural adaptations. People whose disease does not respond to these interventions will progress onto medication once diagnosis is confirmed by sleep specialist services. First line treatment for narcolepsy is methylphenidate. Second line options include dexamfetamine, lisdexamfetamine, modafinil and atomoxetine. Medications are classed as ‘first’ or ‘second’ line based on clinical consensus and can vary across countries. For people whose disease does not respond or people who have considerable adverse effects after first and second line treatments, sodium oxybate is a third line treatment option.[1]

The technology

Pitolisant (Wakix, Bioprojet UK) does not currently have a marketing authorisation in the UK for treating narcolepsy in children and young people aged 6 to 17 years. It has been studied in a clinical trial compared with placebo in children and young people aged 6 to 17 years with narcolepsy, with or without cataplexy. Pitolisant has a UK marketing authorisation in adults for the treatment of narcolepsy with or without cataplexy.

Intervention(s)	Pitolisant
Population(s)	People aged 6 to 17 years with narcolepsy with or without cataplexy
Subgroups	• People aged 6 to 17 years with narcolepsy and cataplexy • People aged 6 to 17 years with narcolepsy without cataplexy
Comparators	Established clinical management without pitolisant, including: • methylphenidate • dexamfetamine • lisdexamfetamine • modafinil • atomoxetine • solriamfetol • sodium oxybate
Outcomes	The outcome measures to be considered include: • excessive daytime sleepiness • number of cataplectic episodes (for those with cataplexy) • adverse effects of treatment • 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. The availability and cost of biosimilar and generic products should be taken into account.
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 technology appraisals:**

	Solriamfetol for treating excessive daytime sleepiness caused by narcolepsy (2022) NICE technology appraisal guidance 758. **Related evidence summary:** Narcolepsy with or without cataplexy in adults: pitolisant (2017) NICE evidence summary 8.
Related National Policy	NHS England (2021) Clinical Commissioning Policy: Sodium oxybate for symptom control of narcolepsy with cataplexy (children and adolescents aged 7 until 19 years) The NHS Long Term Plan (2019) NHS Long Term Plan NHS England (2017) NHS Medicines for Children's Policy NHS England (2023) Manual for prescribed specialist services (2023/2024) Department of Health and Social Care (2016) NHS outcomes framework 2016 to 2017 NHS Digital (2022) NHS Outcomes Framework England, March 2022 Annual Publication

Questions for consultation

Where do you consider pitolisant will fit into the existing care pathway for narcolepsy?

Have all the relevant comparators been included in the scope?

Would pitolisant be a candidate for managed access?

Is solriamfetol used to treat children and young people with narcolepsy in current UK clinical practice?

Are there preferred combinations of medicines for treating children and young people with narcolepsy in current UK clinical practice?

Is cataplexy status expected to affect the clinical or cost-effectiveness of pitolisant for treating children and young people with narcolepsy?

Do you consider that the use of pitolisant can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Pitolisant is currently commissioned to treat adults with narcolepsy with or without cataplexy by local integrated care boards. Would a multiple technology appraisal covering pitolisant for treating narcolepsy in an adult population and a separate children and young people population add value to the NHS?

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

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 pitolisant will be 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. NHS England (2021) Clinical Commissioning Policy: Sodium oxybate for symptom control of narcolepsy with cataplexy (children and adolescents aged 7 until 19 years)

2. NHS (2022) Narcolepsy. Accessed March 2024

3. Narcolepsy UK About Narcolepsy. Accessed March 2024

4. European Medicines Agency (2023) Assessment report: Wakix