Northern Treatment Advisory Group

Northern (NHS) Treatment Advisory Group

Treatment Appraisal: Decision Summary

Date	15th November 2022 (updated)
Appraisal & Details	The Northern (NHS) Treatment Advisory Group reviewed its current recommendation on the use of sodium oxybate (Xyrem®) in the management of narcolepsy with cataplexy in adult patients in light of the RMOC guidance published in October 2019.
Recommendation	**The Northern (NHS) Treatment Advisory Group recommends the use of sodium oxybate in adult patients who have received and benefited from treatment with sodium oxybate as commissioned by NHS England. i.e. continuing treatment for those >19 years old.** The strict NHS England criteria for starting and stopping must continue to be followed. **The Northern (NHS) Treatment Advisory Group also recommends sodium oxybate for use in adults who have not received it as a child as per the RMOC criteria, noting that may sometimes be used in combination with other agents.** **The following criteria for use in adults who have not received sodium oxybate as child apply:** • **Patients presenting with narcolepsy with cataplexy according to International Classification of sleep disorders 3 (ICSD) criteria for Narcolepsy Type 1 AND** • **Patients ≥ 19 years old AND** • **Where patients have co-morbidities, which are also affecting sleep, these should be managed and adequately treated (for example moderate to severe obstructive sleep apnoea or restless legs syndrome) AND** • **Failure to respond to non-pharmacological treatments consisting of behavioural and environmental adaptations, for example planned naps AND** • **Inadequate response (within 3 months) to, or intolerable adverse effects from, or contra-indicated use of, more than one stimulant for narcolepsy, and more than one anticataplectic agent AND** • **Assessed as being able to benefit from sodium oxybate via a specialist sleep centre.** • **Sodium oxybate is generally considered as a final treatment option for patients.** To remain as RED drug (i.e hospital only) as it an ICS commissioned tariff excluded drug. **Assessing need for ongoing treatment** • Patients who show signs of serious adverse events should discontinue therapy. • Improvements in narcolepsy and/ or cataplexy should be determined by expert clinical review, which will include the use of the Epworth Sleepiness Scale and an assessment of symptomatic/quality of life improvements.

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	• Discontinue if there is inadequate response at 3 months for both cataplexy and narcolepsy. Measurements should ideally be compared to scores prior to sodium oxybate treatment (see Appendix 2 for definitions). Expert clinical review and patient history will also contribute to this assessment. • Patients on established therapy should be reviewed at least annually if stable (more frequently if not) to ensure continued benefit. • Trial withdrawal periods can be considered if this is clinically appropriate.
Clinical evidence summary	A meta-analysis and systematic review published in 2012 summarises allof the available randomised controlled trial evidence. The analysis demonstrates significant improvements in number of cataplexy attacks, wakefulness, number of sleep attacks and global clinician global impression of change associated with sodium oxybate against placebo. However, sample sizes for some comparisons were very small, and confidence intervals were wide in several cases, limiting the precision of these estimates of treatment effect. <u>Regional Medicines Optimisation Committee (RMOC) Advisory Statement</u> This statement does not stipulate that sodium oxybate must be commissioned, but aims to assist this decision making process and improve consistency. Adults ≥ 19 years (sodium oxybate treatment naïve): RMOC has suggested some criteria for potentially eligible patients for CCGs to consider funding and commissioning use in this group of patients
Safety	Adverse effects including gastrointestinal effects, dizziness and enuresis were more common with sodium oxybate than placebo. Discontinuation due to adverse effects occurred in 7-9% of patients
Patient Perspective	Narcolepsy is a disabling sleep disorder characterised by excessive daytime sleepiness. Patients are often unable to stay awake or asleep for long periods of time. Around 70% of people with narcolepsy also have cataplexy, which is a sudden loss of muscle tone triggered by strong emotions. Episodes can last from seconds to minutes and occur with varying frequency.
Cost analysis summary	The cost of sodium oxybate varies with the dose, which ranges from 4.5 g to 9g daily. This results in an estimated cost per patient of £6,500 to £13,100 per year. Mean doses used in practice are likely to be in the middle of that range. No cost-effectiveness studies were found. The number of patients eligible for treatment with sodium oxybate is expected to be low. NHS England estimates that there are currently 10 children treated with sodium oxybate nationally. In terms of adults who have not received as a child estimate up to 6 new patients per year. More expensive than other options: • Pitolisant = £3720 to £7740 per year per patient (ex VAT) • Solriamfetol = £2124 to £2976 per year per patient (ex VAT)

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Financial impact PbR: **Excluded.**	ICS commissioned tariff excluded drug for use adults NHSE commissioned tariff excluded drug for use in children and adolescents. Sodium oxybate was protected by several active patents; however generics have started to enter the market in Europe starting in January 2020 which may alter costs. Though Drug Tariff price is based on the original branded product.