FDA Approves Sodium Oxybate to Treat Cataplexy or EDS in Pediatric Patients With Narcolepsy

Key Takeaways

- Sodium oxybate (Lumryz) is now FDA-approved for narcolepsy patients aged 7 and older, addressing cataplexy and excessive daytime sleepiness.
- The extended-release formulation allows for once-at-bedtime dosing, simplifying treatment regimens for patients and caregivers.

Patients aged 7 years and older can now be treated for their cataplexy or excessive daytime sleepiness with the extended-release oral formulation.

The FDA has approved a supplemental new drug application (sNDA) for sodium oxybate (Lumryz; Avadel Pharmaceuticals) for the treatment of cataplexy or excessive daytime sleepiness (EDS) in patients with narcolepsy who are aged 7 years and older. It is an extended-release oral suspension formulation that is taken once at bedtime, which is significant for patients who may adhere to twice-nightly regimens that require waking in the middle of the night to take a second dose.[1]

In May 2023, the treatment was granted by the FDA to treat EDS or cataplexy in adults with narcolepsy, making it the first and only once-at-bedtime treatment for this indication at the time of its approval. Its initial approval was supported by results from the phase 3 REST-ON clinical trial (NCT02720744).[1,2]

Narcolepsy is a chronic neurological condition that impairs the brain's ability to regulate the sleep-wake cycle. This condition affects around 1 in 2000 people in the US, of which approximately 5% are under the age of 18. Once symptom of narcolepsy is EDS, and others can include a sudden loss of muscle tone that is often triggered by cataplexy (or strong emotion), disrupted nighttime sleep, sleep paralysis, and hallucinations when either falling asleep or waking up.[1]

"This approval represents an important milestone for the narcolepsy community, specifically for younger [patients with narcolepsy] and their caregivers who face significant challenges associated with waking up in the middle of the night to complete treatment regimens. With this label expansion, pediatric patients 7 years and older living with narcolepsy now have the same option that adult patients with narcolepsy have—to choose a once-nightly treatment option that does not disrupt sleep for a middle of the night dose," said Greg Divis, CEO of Avadel Pharmaceuticals, in a news release.[1]

The REST-ON clinical trial (NCT02720744) was a double-blind phase 3 trial in which 190 patients with narcolepsy type 1 or 2 aged 16 years and older were randomly assigned to receive either once-nightly sodium oxybate of various doses (4.5 g, 6 g, 7.5 g, or 9 g; n = 97), or placebo (n = 93).[3] The trial's primary end points were maintenance of wakefulness, number of cataplexy attacks, and patients who were "very much" or "much" improved on clinical global impression of improvement from baseline, all of which were assessed at week 14.[4] Secondary end points included polysomnographic measures of sleep stage shifts and nocturnal arousals and patient-reported assessments of sleep quality and refreshing nature of sleep at 6, 7.5, and 9 g; and changes in time spent in each sleep stage, delta power, and assessments in stimulant-use subgroups for prespecified end points.[3]

The findings demonstrated that sodium oxybate was shown to be clinically meaningful and statistically significant in decreasing the number of transitions from wake/N1 to N1, N2, and rapid eye movement (REM) stages, as well as the number of nocturnal arousals. Additionally, sleep quality and refreshing nature of sleep were significantly improved with sodium oxybate compared with placebo. Further, post hoc analyses showed a significant reduction in time spent in N1 and REM as well as increased time spent in N3 with sodium oxybate compared placebo.[3,4]

“The expanded FDA approval for [sodium oxybate] allows me to now share with my patients and their families that there is an FDA-approved treatment that offers a single bedtime dose of medication, provided in a pre-filled packet. I can now offer more options to more patients which allows me to continue my role as a partner in my patients' journeys,” said Anne Marie Morse, DO, a board-certified and fellowship-trained pediatric neurologist and sleep medicine specialist at Geisinger Health System, in the news release.[1]

About the Trial

Trial Name: Once-Nightly Sodium Oxybate for Treatment of Excessive Daytime Sleepiness and Cataplexy in Narcolepsy
ClinicalTrials.gov ID: NCT02720744
Sponsor: Avadel
Completion Date: March 25, 2020

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