NAYZILAM® (midazolam) CIV - Testimony

This Testimony will provide an overview of UCB's product NAYZILAM, the first FDA-approved midazolam intranasal spray.

Before discussing product, it is important to introduce the burden of seizure clusters and the significant unmet need for an intranasal rescue therapy.

- In the US, 1 in 26 patients will develop epilepsy in their lifetime, 3.4 million Americans are living with epilepsy.[1,2]
- Patients who experience seizure clusters represent ~5% of the total epilepsy population, or a total of 150k-200k patients in the US. [2-4]
- For perspective, Nevada has 31,600 residents currently living with epilepsy, therefore you could anticipate 1,580 to have seizure clusters.[2]
- 30-40% of seizure cluster patients utilized the ER over a one-year period, and the odds of seizure related hospitalizations (not related to SE) for seizure cluster patients was 5x the odds for non-seizure cluster patients.[5-7]

Despite the availability of an FDA-approved treatment, unmet needs remain in this group of patients. Less than 10% of seizure cluster patients use the FDA-approved diazepam rectal gel.[3]

- The urgent need to treat seizure clusters with a rescue medication is well established in the medical literature.[8,9]
- Underutilization of rescue therapy leads to increased use of emergency care, and 26% of patients report seeking emergency care as their first response for managing seizure.[6]

For decades, UCB has been committed to developing new medicines and solutions to address the unmet needs for people with epilepsy.

Approved by the FDA in May 2019, NAYZILAM (midazolam intranasal spray) is a benzodiazepine indicated for the acute treatment of intermittent, stereotypic episodes of frequent seizure activity (i.e., seizure clusters, acute repetitive seizures) that are distinct from a patient's usual seizure pattern in patients with epilepsy 12 years of age and older. NAYZILAM is a Schedule IV controlled substance. [10]

NAYZILAM has demonstrated efficacy in stopping seizure clusters in a phase 3, double-blind, placebo-controlled study of 292 patients 12 years of age or more, with seizure clusters.[11]

The open-label extension trial of 161 seizure cluster patients 12 years of age or older reported sustained efficacy after repeated, intermittent, acute treatment with NAYZILAM.[12]

- 87.6% of patients had seizure termination within 10 minutes of treatment with NAYZILAM and 69% had no seizure recurrence within 6 hours. [12]
- The median time to documented return to full baseline functionality was 1.2 hours (0.5-3.0 hours). [12]
- Overall, 39.9% of seizure clusters required a second dose of NAYZILAM. Of 769 seizure cluster episodes, 80.2% (95% CI = 77.2-83.0) met the criteria for treatment success with the second dose. [12]
- By patient, 81.64% of cluster episodes met the criteria for treatment success following all doses (one or two) of NAYZILAM. Efficacy did not diminish over time with the number of treated episodes. [12]

RISKS FROM CONCOMITANT USE WITH OPIOIDS[10]
Concomitant use of benzodiazepines, including NAYZILAM, and opioids may result in profound sedation, respiratory depression, coma, and death.
o Reserve concomitant prescribing of these drugs for use in patients for whom alternative treatment options are inadequate.
o Limit dosages and durations to the minimum required.
o Follow patients for signs and symptoms of respiratory depression and sedation

For detailed information on NAYZILAM's safety profile, including important warnings and precautions, please refer to the Package Insert. NAYZILAM is contraindicated in patients with acute narrow-angle glaucoma. The most common adverse reactions are somnolence, headache, nasal discomfort, throat irritation, and rhinorrhea.[10]

- Reports of somnolence were 4% in the placebo group compared to 10% for NAYZILAM. A second dose of NAYZILAM did not cause an increase in reports of somnolence compared to patients receiving a single 5mg dose.[10]
- Long term NAYZILAM safety data was obtained in the phase 3, open label extension trial, with 1998 seizure clusters treated in 161 patients (mean number of clusters per patient of 12.4, range 1-73). NAYZILAM was generally well tolerated.[12]

NAYZILAM is supplied in a single-dose nasal spray unit, that delivers 5mg of midazolam in 0.1mL of solution. Each unit is individually packed in a blister, and each box of NAYZILAM contains 2 blisters.[10]

Please provide access to NAYZILAM for Medicaid patients suffering from seizure clusters.

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