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TASIMELTEON IMPROVES SLEEP QUALITY AND BEHAVIOR IN INDIVIDUALS WITH SMITH-MAGENIS SYNDROME (SMS) IN AN OPEN-LABEL STUDY

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Introduction: Individuals diagnosed with Smith-Magenis Syndrome (SMS), a rare genetic disorder due to a deletion on chromosome 17, typically exhibit self-injurious and aggressive behaviors and disrupted nighttime sleep (i.e., difficulties falling asleep or frequent, prolonged nighttime awakenings). The associated nighttime sleep disturbances may be related to the observed inappropriate timing of the endogenous melatonin secretion during the daytime in this population.

Materials and methods: There were 12 individuals (7 male) diagnosed with SMS, ages 16-38 years (mean + SD = 23.7 ± 7.3 years), who were assessed during a ~6-week baseline phase followed by an open-label treatment phase (tasimelteon, 20 mg capsule, nightly 1 h prior to bedtime) for 9-36 weeks. Parents rated their child's nighttime sleep quality (1=Poor to 5= Excellent) every morning and rated their child's behavior using the Aberrant Behavior Checklist-Community (ABC-C), a 58-item checklist with a 4-point rating scale (0=“not at all a problem” to 3= “the problem is severe in degree”), every 3-6 weeks during both phases of the study. A paired Student´s t-test was used to analyze the data.

Results: In comparison to the baseline phase (mean change; pvalue), scores of parent-reported sleep quality significantly increased (+0.51; 0.0105) and total score on the ABC-C significantly decreased (-15.89; 0.0006) during treatment phase, including four ABC-C subscales, “Hyperactivity, Noncompliance” (-5.96; 0.0049), “Irritability, Agitation, Crying” (-5.62; 0.0004), “Lethargy” (-2.10; 0.0384) and “Stereotypic Behavior” (-1.24; 0.0049). The remaining ABC-C subscale, “Inappropriate Speech”, exhibited a decreasing trend (-0.96; 0.0754).

Conclusions: Parents of children with SMS reported improvement in sleep quality and decrease in aberrant behaviors during treatment with tasimelteon, as compared to baseline. Although not collected under placebo-controlled conditions, these data suggest that nightly tasimelteon treatment may alleviate the nighttime sleep disruption in individuals diagnosed with SMS and may impact daytime aberrant behaviors.

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