Hetlioz®, Hetlioz LQ™ (tasimelteon) – New orphan indication, new formulation approval

- On December 1, 2020, Vanda Pharmaceuticals announced the FDA approval of Hetlioz (tasimelteon) capsules, for the treatment of nighttime sleep disturbances in Smith-Magenis Syndrome (SMS) in patients 16 years of age and older, and Hetlioz LQ oral suspension for the treatment of nighttime sleep disturbances in SMS in pediatric patients 3 to 15 years of age.

- Hetlioz was previously only available as a capsule formulation and approved for Non-24-Hour Sleep-Wake Disorder (Non-24).

- SMS is a developmental disorder affecting about 1 in 15,000 to 25,000 births in the U.S. Patients with SMS present with a number of physical, mental and behavioral problems. The most common symptom of SMS is a severe sleep disorder associated with significant disruption in the lives of patients and their families.

- The approval of Hetlioz and Hetlioz LQ for the new indication was based on a 9-week, double-blind, placebo-controlled, crossover study in 25 adults and pediatric patients with SMS. The primary endpoints were nighttime total sleep time and nighttime sleep quality from a parent/guardian-recorded diary. Nighttime total sleep time was reported as a time unit in hours and minutes. Nighttime sleep quality was rated as follows: 5 = excellent; 4 = good; 3 = average; 2 = fair; 1 = poor. In accordance with the cross-over design, the efficacy comparisons were within patient.

    — Compared to placebo, treatment with Hetlioz resulted in a statistically significant improvement in the 50% worst nights' sleep quality. Although improvement on the 50% worst total nighttime sleep time numerically favored Hetlioz treatment, the difference was not statistically significant.

- The recommended dosage of Hetlioz capsules in patients 16 years and older is 20 mg orally once daily. The recommended dosage of Hetlioz LQ oral suspension in pediatric patients 3 years to 15 years of age is based on body weight; for patients ≤ 28 kg, the daily dose is 0.7 mg/kg and for patients > 28 kg, the daily dose is 20 mg. Hetlioz and Hetlioz LQ should be administered one hour before bedtime, at the same time every night.

    — Hetlioz capsules and Hetlioz LQ oral suspension are not substitutable.
    — Refer to the Hetlioz drug label for dosing for Non-24.