



## Hetlioz<sup>®</sup>, Hetlioz LQ<sup>™</sup> (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  $\leq$  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.



OptumRx<sup>®</sup> specializes in the delivery, clinical management and affordability of prescription medications and consumer health products. We are an Optum<sup>®</sup> company — a leading provider of integrated health services. Learn more at [optum.com](https://www.optum.com).

All Optum<sup>®</sup> trademarks and logos are owned by Optum, Inc. All other brand or product names are trademarks or registered marks of their respective owners.

This document contains information that is considered proprietary to OptumRx and should not be reproduced without the express written consent of OptumRx.

RxNews<sup>®</sup> is published by the OptumRx Clinical Services Department.

©2020 Optum, Inc. All rights reserved.