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US FDA approves first treatment for rare genetic disorder Prader-Willi syndrome

By **Sneha S K**

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[1/2] The corporate logo of the U.S. Food and Drug Administration (FDA) is shown in Silver Spring, Maryland, November 4, 2009. REUTERS/Jason Reed/File Photo Purchase Licensing Rights

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FDA approves first treatment to treat insatiable hunger

Vykat XR to be available in US from April 2025 — Soleno

March 26 (Reuters) - The U.S. Food and Drug Administration on Wednesday approved Soleno Therapeutics' (SLNO.O) drug to treat a rare genetic disorder, making it the first treatment available for patients who experience feelings of intense and persistent hunger.

The drug, to be called Vykat XR, treats hyperphagia, the hallmark symptom of the Prader-Willi syndrome, a genetic disorder caused by full or partial deletions on chromosome 15 that affect the regulation of gene expression, or how genes turn on and off.

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The debilitating condition, which affects about 50,000 people in the U.S., impacts everyday aspects of life, such as eating, behavior and mood.

Starting in April 2025, Soleno plans to make the drug available in the U.S. for patients aged four years and older who have PWS with hyperphagia. The drug will have an annual average cost of $466,200 and will be dosed as per patients' weight, the company said.

The syndrome's symptoms appear immediately, with infants born with low muscle tone and an inability to breastfeed. As patients grow older, children develop hyperphagia, a feeling of insatiable hunger persisting even after meals. This could occur as early as age four but, on average, starts at about age eight and then continues into adulthood.

The increased appetite can lead to rapid weight gain and, if not controlled, obesity and related problems such as respiratory difficulties and heart disease. The company estimates the average lifespan of people with the disease is between 21 and 29 years.

The drug is a once-daily oral pill designed to target a specific pathway in the brain to help reduce hyperphagia by decreasing the secretion of a peptide that is known to regulate appetite.

LONG-AWAITED APPROVAL

The long-awaited approval comes after the FDA's extended review of the drug and mixed results from a late-stage trial on its efficacy. The approval was based on data from the study and a randomized withdrawal study.

In the late-stage trial involving 127 patients, the drug helped reduce feelings of intense hunger in the group of patients who had severe hyperphagia. The drug, however, did not show a statistically significant change in hyperphagia compared to the placebo group in the study.

A long-term follow-up study in patients who had completed the late-stage trial showed the drug significantly reduced hyperphagia after at least one year of treatment.

Justice Faith, a 21-year-old from Minneapolis, Minnesota, with PWS, said she is "very excited" that a treatment option may be available soon for hyperphagia. "My experience with hyperphagia, like everyone with PWS, is different from others," she said in an emailed statement ahead of the approval.

An increased interest and awareness about food, as well as a generalized increase in anxiety, develop in people with PWS around childhood. Hyperphagia can also lead to a range of disruptive food-related behaviors, including aggressive food seeking.

"There are a lot of questions about food, 'When are we going to have lunch? Are you sure it's time for lunch?' It's just non-stop," said Jennifer Miller, professor of pediatric endocrinology at the University of Florida. She was also a principal investigator on the trials for Vykat XR.

"One of the patients, when I first started doing this 26 years ago, said to me, 'Imagine the hungriest you've ever been. That's how I feel all the time. I never ever feel like I've eaten.' That's why I started (treating patients), because of that comment," Miller said.

Anthony Holland, president of the International Prader-Willi Syndrome Organisation said the IPWSO will work closely with the company as well as other global health bodies to expand access to the drug worldwide. "One of the tasks of our organization will be to campaign for its use more widely," Holland said.

Reporting by Sneha S K and Bageshri Banerjee in Bengaluru; Editing by Krishna Chandra Eluri and Alan Barona

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