FDA Approves Novel Non-Opioid Treatment for Moderate to Severe Acute Pain

First Drug Approved in New Class of Non-Opioid Pain Medicines; Agency Continues to Take Steps to Support New Approaches for Pain Management

Today, the U.S. Food and Drug Administration approved Journavx (suzetrigine) 50 milligram oral tablets, a first-in-class non-opioid analgesic, to treat moderate to severe acute pain in adults. Journavx reduces pain by targeting a pain-signaling pathway involving sodium channels in the peripheral nervous system, before pain signals reach the brain.

Journavx is the first drug to be approved in this new class of pain management medicines.

Pain is a common medical problem and relief of pain is an important therapeutic goal. Acute pain is short-term pain that is typically in response to some form of tissue injury, such as trauma or surgery. Acute pain is often treated with analgesics that may or may not contain opioids.

The FDA has long supported development of non-opioid pain treatment. As part of the FDA Overdose Prevention Framework, the agency has issued draft guidance aimed at encouraging development of non-opioid analgesics for acute pain and awarded cooperative grants to support the development and dissemination of clinical practice guidelines for the management of acute pain conditions.

"Today's approval is an important public health milestone in acute pain management," said Jacqueline Corrigan-Curay, J.D., M.D., acting director of the FDA's Center for Drug Evaluation and Research. "A new non-opioid analgesic therapeutic class for acute pain offers an opportunity to mitigate certain risks associated with using an opioid for pain and provides patients with another treatment option. This action and the agency's designations to expedite the drug's development and review underscore FDA's commitment to approving safe and effective alternatives to opioids for pain management."

The efficacy of Journavx was evaluated in two randomized, double-blind, placebo- and active-controlled trials of acute surgical pain, one following abdominoplasty and the other following bunionectomy. In addition to receiving the randomized treatment, all participants in the trials with inadequate pain control were permitted to use ibuprofen as needed for "rescue" pain medication. Both trials demonstrated a statistically significant superior reduction in pain with Journavx compared to placebo.

The safety profile of Journavx is primarily based on data from the pooled, double-blind, placebo- and active-controlled trials in 874 participants with moderate to severe acute pain following abdominoplasty and bunionectomy, with supportive safety data from one single-arm, open-label study in 256 participants with moderate to severe acute pain in a range of acute pain conditions.

The most common adverse reactions in study participants who received Journavx were itching, muscle spasms, increased blood level of creatine phosphokinase, and rash. Journavx is contraindicated for concomitant use with strong CYP3A inhibitors. Additionally, patients should avoid food or drink containing grapefruit when taking Journavx.

The application received Breakthrough Therapy, Fast Track and Priority Review designations by the FDA.

The FDA granted approval of Journavx to Vertex Pharmaceuticals Incorporated.

FDA News released Jan 30, 2025. www.fda.gov.

FDA Approves First Rapid-Acting Insulin Biosimilar Product for Treatment of Diabetes

Agency Continues Efforts to Increase Access to Insulin Treatment Options

Today, the U.S. Food and Drug Administration approved Merilog (insulin-aspart-szjj) as biosimilar to Novolog (insulin aspart) for the improvement of glycemic control in adults and pediatric patients with diabetes mellitus. Merilog, a rapid-acting human insulin analog, is the first rapid-acting insulin biosimilar product approved by the FDA. As a rapid-acting insulin, Merilog helps to lower mealtime blood sugar spikes to improve control of blood sugar in people with diabetes. The approval is for both a 3 milliliter (mL) single-patient-use prefilled pen and a 10 milliliter (mL) multiple-dose vial.

Merilog is the third insulin biosimilar product approved by the FDA and joins the two long-acting insulin biosimilar products approved in 2021 by the FDA. Approval of biosimilar products can increase patient access to safe and effective treatment options.

"The FDA has now approved three biosimilar insulin products to treat diabetes," said Peter Stein, M.D., director of the Office of New Drugs in the FDA's Center for Drug Evaluation and Research. "Today's approval highlights our continued efforts to improve the efficiency of the biosimilar approval process to help support a competitive marketplace and increase options for costly treatments, like insulin. Increasing access to safe, effective and high-quality medications at potentially lower cost remains a continued priority for the FDA."

Biological products include medications for treating many serious illnesses and chronic health conditions, including diabetes. A biosimilar is a biological product that is highly similar to, and has no clinically meaningful differences from, a biological product already approved by the FDA (also called the reference product). Patients can expect the same safety and effectiveness from the biosimilar as from the reference product. To date, the FDA has approved 65 biosimilar products for a variety of health conditions.

More than 38 million people in the U.S. have been diagnosed with diabetes, a disease that occurs when blood glucose (sugar) is too high. Approximately 8.4 million Americans rely on insulin therapy, either rapid-acting and/or long-acting, to manage diabetes. Insulin, a hormone made by the pancreas, helps glucose get into a person's cells to be used for energy. With diabetes, the pancreas doesn't make enough insulin to keep blood sugar levels in the normal range, which can lead to serious health problems for patients.

"For the millions of people who rely on daily injections of insulin for treatment of diabetes, having a biosimilar option for their rapid-acting insulin injection can truly make a difference, as biosimilar products have the potential to increase access to these life-saving medications," said Sarah Yim, M.D., director of the Office of Therapeutic Biologics and Biosimilars in the FDA's Center for Drug Evaluation and Research.

Like Novolog, Merilog should be administered within five to ten minutes prior to the start of a meal. Merilog is administered subcutaneously (under the skin) by injection into the stomach, buttocks, thighs or upper arms. Dosing of Merilog should be individualized and adjusted based on the patient's needs.

Merilog may cause serious side effects, including hypoglycemia (low blood sugar), severe allergic reactions and hypokalemia (low potassium in blood).

Other common side effects may include injection site reactions, itching, rash, lipodystrophy (skin thickening or pitting at the injection site), weight gain and swelling of hands and feet.

The FDA granted approval of Merilog to Sanofi-Aventis U.S. LLC.

FDA News released Feb 14, 2025. www.fda.gov.

FDA Approves First Treatment for Cerebrotendinous Xanthomatosis, a Rare Lipid Storage Disease

Today, the U.S. Food and Drug Administration approved Ctexli (chenodiol) for the treatment of cerebrotendinous xanthomatosis (CTX) in adults. Ctexli is the first FDA-approved drug to treat CTX, a very rare lipid storage disease.

"The FDA is dedicated to supporting new drug development for rare diseases including very rare metabolic diseases like cerebrotendinous xanthomatosis," said Janet Maynard, M.D., M.H.S., director of the Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine, in the FDA's Center for Drug Evaluation and Research. "CTX is a progressive multisystemic disorder that significantly impacts patients and previously lacked approved treatments. Today's approval provides a safe and effective treatment option for CTX."

CTX is a genetic metabolic disorder caused by a mutation in a gene called CYP27A1 resulting in a deficiency of the enzyme that is important in the body's ability to break down fats. Due to reduced bile acid production in the liver, patients with CTX are unable to break down cholesterol in a normal way, resulting in deposition of atypical cholesterol metabolites (substances that result from the breakdown of cholesterol) in various places in the body including the brain, liver, skin and tendons, leading to damage to those organs and tissues. Ctexli works to replace deficient levels of one of the bile acids, reducing the abnormal deposits of cholesterol metabolites thought to be responsible for clinical abnormalities in CTX.

The efficacy of Ctexli for the treatment of patients with CTX was evaluated in a double-blind, placebo controlled, randomized crossover withdrawal trial. The 24-week trial demonstrated that treatment with Ctexli, 250 milligrams three times per day, resulted in significant reduction in plasma cholestanol and urine 23S-pentol (cholesterol metabolites that are

markedly increased in CTX patients) compared to placebo treatment.

The prescribing information for Ctexli includes a warning for liver toxicity in all patients with increased risk for liver damage in patients with pre-existing liver disease or bile duct abnormalities. Patients should obtain liver blood tests before starting treatment, annually while on treatment and as clinically indicated. If signs of liver toxicity (e.g., stomach pain, nausea, fatigue, dark urine, bruising, yellowing of the eyes and skin, itching) occur, patients are advised to see their doctor and discontinue Ctexli.

The most common side effects of Ctexli are diarrhea, headache, abdominal pain, constipation, hypertension, muscular weakness and upper respiratory tract infection.

The recommended dosage is 250 milligrams, taken orally three times a day.

The FDA granted Ctexli Priority Review, Fast Track and Orphan Drug designations for this application.

The approval of Ctexli was granted to Mirum Pharmaceuticals Inc.

FDA News released Feb 21, 2025. www.fda.gov.

Source: FDA