

Orladeyo™ (berotralstat) – New orphan drug approval

- On December 3, 2020, [BioCryst Pharmaceuticals announced](#) the FDA approval of [Orladeyo \(berotralstat\)](#), for prophylaxis to prevent attacks of hereditary angioedema (HAE) in adults and pediatric patients 12 years of age and older.
 - The safety and effectiveness of Orladeyo for the treatment of acute HAE attacks have not been established. Orladeyo should not be used for treatment of acute HAE attacks.
 - Additional doses or doses of Orladeyo higher than 150 mg once daily are not recommended due to the potential for QT prolongation.
- HAE is a rare inherited disorder affecting an estimated [one in 50,000 to 150,000](#) individuals worldwide. It is characterized by recurrent episodes of the accumulation of fluids outside of the blood vessels, blocking the normal flow of blood or lymphatic fluid. It can cause swelling and obstruction of the airway, a potentially very serious complication.
- Orladeyo, a plasma kallikrein inhibitor, is the first orally administered non-steroidal option for preventing HAE attacks.
- The efficacy of Orladeyo was established in a randomized, double-blind, placebo-controlled, study in 120 patients 12 years of age and older with HAE. Patients were randomized into 1 of 3 treatment arms, stratified by baseline attack rate: berotralstat 110 mg, berotralstat 150 mg, or placebo once daily, for the 24-week treatment period. The primary endpoint was the rate of HAE attacks.
 - Orladeyo 110 mg provided a 30.0% rate reduction in HAE attacks vs. placebo (95% CI: 4.6, 48.7, p = 0.024) and Orladeyo 150 mg provided a 44.2% rate reduction vs. placebo (95% CI: 23.0, 59.5; p < <0.001).
 - Reductions in attack rates were observed in the first month of treatment with Orladeyo 150 mg and 110 mg and were sustained through 24 weeks.
- A warnings and precaution for Orladeyo is risk of QT prolongation with higher-than-recommended dosages.
- The most common adverse reactions (≥ 10%) with Orladeyo use were abdominal pain, vomiting, diarrhea, back pain, and gastroesophageal reflux disease.
- The recommended dose of Orladeyo is one 150 mg capsule taken orally once daily with food.
- The list price for Orladeyo is expected to be [\\$485,000](#) per patient per year.
- BioCryst Pharmaceuticals plans to launch Orladeyo in December 2020. Orladeyo will be available as 110 mg and 150 mg capsules.