

Patients for Bitopertin Launches, Calls for Urgent Path Forward After FDA Delays Approval Decision

New patient-driven initiative amplifies the voices of people living with EPP and XLP, two rare diseases in which sunlight can cause severe phototoxic pain

BETHESDA, MD, UNITED STATES, April 9, 2026 /EINPresswire.com/ -- [Patients for Bitopertin](#), an independent patient-driven advocacy effort, today announced the launch of a new public campaign to elevate the voices of people living with the rare diseases [erythropoietic protoporphyria \(EPP\)](#) and [X-linked protoporphyria \(XLP\)](#) and to call attention to the urgent need for a timely path forward for bitopertin.



With EPP and XLP, painful reactions to sunlight can begin within minutes of sun exposure

The effort follows the U.S. Food and Drug Administration’s (FDA) February 13, 2026 Complete Response Letter (CRL) for bitopertin, an investigational therapy being studied for EPP and XLP. In the CRL, the FDA acknowledged that bitopertin significantly lowered whole blood metal-free protoporphyrin IX, an important disease biomarker, but said it wanted additional evidence linking those reductions to meaningful clinical benefit, including more pain-free time in the sun. A [Disc Medicine press release](#) shared that the issues raised were addressable and not related to chemistry, manufacturing, or controls – and that the drug has a strong safety profile. The company has said the Phase 3 APOLLO study, required for this data, anticipates top-line data in the fourth quarter of 2026.

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Ginger Gonsalves, EPP Patient

For people living with EPP and XLP, this path to approval represents a significant delay. These ultra-rare diseases make even short exposure to visible light, including sunlight, painful and life-

limiting. Patients and families are forced to structure daily life around light avoidance, constant protection, and the steady loss of everyday experiences.

“Patients with EPP and XLP do not have time to wait,” said Ginger Gonsalves, EPP patient and spokesperson for Patients for Bitopertin. “For our community, every delay means more pain, more isolation, and more life on hold. Patients for Bitopertin was created to make sure the reality of life with this disease is seen, heard, and impossible to ignore.”

Patients for Bitopertin is inviting patients, families, caregivers, friends, and supporters to take action by sharing personal stories, posting on social media, contacting legislators, sending messages to FDA leadership.

The initiative also launches with a dedicated website designed to serve as a hub for patient stories, media coverage, advocacy actions, and resources related to the current situation with bitopertin.

At a time when the FDA has expressed interest in innovation and commonsense pathways for rare disease therapies, Patients for Bitopertin hopes this effort will encourage leadership to find a path forward for this community.

To learn more, share your story, or get involved, visit: www.patientsforbitopertin.org

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PATIENTS FOR BITOPERTIN

Patients for Bitopertin



Before and after starting bitopertin, EPP patient Craig Leppert can now spend time in the sun without pain.

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