Atlas Molecular Pharma Announces First Three Patients with Congenital Erythropoietic Porphyria Dosed with ATL-001

On October 19, 2026, the first patients in the Phase 1/2 clinical trial ATL001-PII-CEP received their first dose and will continue on a daily oral dose over the coming months.

Derio (Bizkaia), Spain, October 20, 2026

On October 19, 2026, the first three patients with congenital erythropoietic porphyria (CEP) enrolled in the ATL001-PII-CEP clinical trial received their first dose of ATL-001, Atlas Molecular Pharma's investigational oral drug for CEP. This is the first time ATL-001 has been given to people with this severe, ultra-rare disease, for which no drug is specifically approved.

The three patients started treatment after completing the initial observation phase (*natural history*). Over the coming months, they will continue taking a daily dose of ATL-001 oral solution.

"Dosing these first three patients with ATL-001 is a very important moment for the whole Atlas team and a meaningful step for CEP research. We have worked for years to get here, and we want to thank the patients and their families for their trust and commitment, which make it possible for this study to move forward. We will continue to work with rigor, caution, and transparency," said Susana Castel, CEO of Atlas Molecular Pharma.

The trial

ATL001-PII-CEP is an open-label, prospective Phase 1/2 trial with an N-of-1 design (each patient is assessed individually). It will study the safety and efficacy of ATL-001 in adults with CEP. Approximately six patients are expected to take part, each receiving daily treatment for about 48 weeks.

In 2024, ATL-001 completed a Phase 1 trial in healthy volunteers that evaluated its safety, tolerability, and pharmacokinetics. This Phase 1/2 trial will determine whether the treatment is safe and well tolerated in patients with CEP.

The study will also assess changes in skin lesions caused by photosensitivity, other clinical signs and symptoms, laboratory parameters, and participants' quality of life.

The trial is being conducted at hospitals in the United States, with internationally recognized CEP investigators: Dr. Robert Desnick, principal investigator, at the Icahn School of Medicine at Mount Sinai (New York); Dr. Angelika Erwin at Cleveland Clinic (Ohio); and Dr. Hetanshi Naik at Stanford University School of Medicine (California).

Congenital erythropoietic porphyria and ATL-001

Congenital erythropoietic porphyria (CEP) is an ultra-rare, severe, and highly debilitating genetic disease for which there are currently no specifically approved drugs. It is caused by deficient activity of the enzyme uroporphyrinogen III synthase (UROS), which is essential for heme biosynthesis. This defect leads to the accumulation of porphyrins and can cause anemia, severe skin lesions, pain, and extreme sensitivity to sunlight.

ATL-001 is a pharmacological chaperone designed to stabilize the altered UROS enzyme and increase its functional activity, with the aim of reducing porphyrin accumulation and,

potentially, the clinical manifestations of the disease. ATL-001 is currently the only drug candidate in clinical development specifically for CEP.

Regulatory support

ATL-001 has held orphan designation in Europe (orphan medicinal product, OMP) and in the United States (orphan drug designation, ODD) since 2018. In 2025, the U.S. Food and Drug Administration (FDA) granted ATL-001 Fast Track designation and included it in its Rare Disease Endpoint Advancement (RDEA) pilot program.

In addition, Dr. Robert Desnick is the principal investigator of a US$2.5 million grant awarded by the FDA's Office of Orphan Products Development (OOPD) to the participating hospitals to fund the Phase 2 clinical development of ATL001-PII-CEP.

About Atlas Molecular Pharma

Atlas Molecular Pharma is a biotechnology company spun off from CIC bioGUNE and based in the Bizkaia Science and Technology Park (Spain). It develops therapies for rare and ultra-rare diseases. Its portfolio includes programs based on pharmacological chaperones and other therapeutic approaches for rare and ultra-rare diseases. Atlas works under an open innovation model in collaboration with academic centers and industry partners, and is backed by partners and investors including Inveready, CDTI, Ezten FCR, and Kereon Partners.

www.atlasmolecularpharma.com

Contact

Susana Castel, CEO: scastel@atlasmolecularpharma.com

Oscar Millet, Fundador y CSO – oscar@atlasmolecularpharma.com