

News Release

Not intended for UK Media

BlueRock Therapeutics' investigational cell therapy lemiretprocel (OpCT-001) for primary photoreceptor diseases granted orphan drug designations by FDA and EMA

- U.S. FDA designations are for treating retinitis pigmentosa and cone-rod dystrophy
- EU EMA designations are for treating syndromic and non-syndromic inherited retinal disorders (IRDs) of the rod-dominant phenotype and non-syndromic IRDs of the cone-dominant phenotype
- Lemiretprocel (OpCT-001) is an iPSC-derived investigational cell therapy being clinically tested in a Phase 1/2a study for the treatment of retinitis pigmentosa, Usher syndrome, and cone-rod dystrophies

Berlin, Germany, and Cambridge, MA, USA September 30, 2026 – Bayer AG and its wholly owned, independently operated subsidiary BlueRock Therapeutics LP, a clinical stage cell therapy company, today announced that its investigational cell therapy, lemiretprocel (OpCT-001), has received orphan drug designations from the U.S. FDA and the EU EMA. U.S. FDA designations are for treating retinitis pigmentosa and cone-rod dystrophy. EU EMA designations are for treating both syndromic and non-syndromic inherited retinal disorders (IRDs) of the rod-dominant phenotype and non-syndromic IRDs of the cone-dominant phenotype.

Lemiretprocel (OpCT-001) is an investigational induced pluripotent stem cell (iPSC)-derived cell therapy being tested in a Phase 1/2a clinical study (CLARICO) for the treatment of primary photoreceptor diseases, which are a subgroup of inherited retinal disorders that include retinitis pigmentosa, Usher syndrome, and cone-rod dystrophy.¹ Lemiretprocel (OpCT-001) aims to restore vision in people living with primary photoreceptor diseases by replacing lost cells in the retina with new functional cells.

“Receiving these designations from the U.S. FDA and the EU EMA is a notable step forward for lemiretprocel,” said Amit Rakhit, MD, MBA, BlueRock’s Chief Medical Officer. “We believe that lemiretprocel has great promise as a potential therapeutic option for restoring vision in people living with primary photoreceptor diseases and look forward to continuing to work with health authorities on its clinical development.”

“Together with BlueRock, we are progressing the first-ever clinical trial for an iPSC-derived cell therapy in primary photoreceptor diseases,” said Christian Rommel, Executive Vice President and Global Head of Research and Development of the Pharmaceuticals Division at Bayer. “Receiving orphan drug designations from both the FDA and EMA recognizes lemiretprocel’s potential to address a wide range of inherited retinal disorders for patients that currently have very limited treatment options.”

Lemiretprocel (OpCT-001) is an investigational cell therapy that has not been approved by any regulatory authority, and its efficacy and safety have not been established or fully evaluated. Lemiretprocel is the International Nonproprietary Name for OpCT-001 as acknowledged by the World Health Organization.²

¹<https://www.ncbi.nlm.nih.gov/books/NBK519518>;
<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC1808442>;
<https://www.nidcd.nih.gov/health/usher-syndrome#b>

²[https://cdn.who.int/media/docs/default-source/international-nonproprietary-names-\(inn\)/pl134.pdf?sfvrsn=87a89aba_3&download=true](https://cdn.who.int/media/docs/default-source/international-nonproprietary-names-(inn)/pl134.pdf?sfvrsn=87a89aba_3&download=true)

About the CLARICO study

CLARICO is a Phase 1/2a first-in-human, multisite, 2-part interventional study to evaluate the safety, tolerability, and the effect on clinical outcomes of OpCT-001 in up to approximately 54 adults with primary photoreceptor disease. Phase 1 will focus on safety and features a dose-escalation design. Phase 2 is designed to gather additional safety data and assess the effect of OpCT-001 on measures of visual function, functional vision, and anatomic measures of engraftment in different clinical subgroups. Phase 1 will include 4 planned dose levels to be administered across 4 cohorts (Cohorts 1 through 4). Dose escalation in Phase 1 will be conducted using a standard 3+3 scheme in which a total of 12 to 24 legally blind participants (~3 to 6 per cohort) will receive escalating doses of OpCT-001 after acceptable safety signals with the prior lower dose. Phase 2 is planned to enroll a maximum of 30 participants across 2 cohorts (Cohorts 5 and 6) to evaluate 2

dose levels of OpCT-001 that will be selected based on Phase 1 safety and tolerability data. Phase 2 participants will be randomized 1:1 to either Cohort 5 or Cohort 6. Phase 2 participants and the investigator/study site personnel outside of the surgical team will be masked to OpCT-001 dose assignments. Further details of the trial can be found at www.clinicaltrials.gov (NCT06789445).

About BlueRock Therapeutics LP

BlueRock Therapeutics LP is a clinical stage cell therapy company focused on creating cellular medicines to treat devastating diseases. We are harnessing the power of cell therapy to create a pipeline of new medicines for people suffering from neurological and ophthalmic diseases. Two of our novel investigational cell therapies, bemdaneprocel (BRT-DA01) for the treatment of Parkinson's disease and OpCT-001 for the treatment of primary photoreceptor diseases are clinical stage programs. Bemdaneprocel has RMAT (Regenerative Medicine Advanced Therapy) and Fast Track designation from the U.S. FDA (Food and Drug Administration) and is being tested in a Phase 3 clinical trial, exPDite-2. OpCT-001 has Fast Track designation from the FDA and is being tested in a Phase 1/2a clinical trial, CLARICO. BlueRock was founded in 2016 as a joint venture of Versant Ventures and Leaps by Bayer, the impact investing arm of Bayer AG that invests in paradigm-shifting breakthrough innovation. In late 2019, BlueRock became a wholly owned, independently operated subsidiary of Bayer AG. Our culture is defined by the courage to persist regardless of the challenge, the urgency to transform medicine and deliver hope, integrity guided by mission, and community-mindedness with the understanding that we are all part of something bigger than ourselves. For more information, visit www.bluerocktx.com.

About Bayer

Bayer is a global enterprise with core competencies in the life science fields of health care and nutrition. In line with its mission, "Health for all, Hunger for none," the company's products and services are designed to support efforts to master major challenges presented by a growing and aging global population and create value through innovation and growth. The Bayer brand stands for trust, reliability and quality throughout the world. In fiscal 2025, the Group employed around 88,000 people and had sales of 45.6 billion euros. R&D expenses amounted to 5.8 billion euros. For more information, go to www.bayer.com.

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Forward-Looking Statements BlueRock Therapeutics

Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may be identified by the use of forward-looking words such as “anticipate,” “believe,” “forecast,” “estimate,” “plan,” and “intend,” among others. These forward-looking statements are based on BlueRock’s current expectations and actual results could differ materially. There are a number of factors that could cause actual events to differ materially from those indicated by such forward-looking statements. These factors include, but are not limited to, the initiation, timing, progress, activities, goals and reporting of results of any preclinical programs and clinical trials and research and development programs, the potential benefits, timing and future operation of the agreements with FUJIFILM Cellular Dynamics and Opsi Therapeutics, the ability to advance therapies into, and successfully initiate, enrol and complete clinical trials, the potential clinical utility of product candidates, the regulatory pathway of, and the timing or likelihood of any regulatory filings and approvals for, any product candidates, and the ability to, and extent of, potentially commercializing any product candidate, are forward looking. As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. Except as expressly required by law, BlueRock does not undertake an obligation to update or revise any forward-looking statement. All of BlueRock’s forward-looking statements are expressly qualified by all such risk factors and other cautionary statements. The information set forth herein speaks only as of the date hereof.

Forward-Looking Statements

This release may contain forward-looking statements based on current assumptions and forecasts made by Bayer management. Various known and unknown risks, uncertainties and other factors could lead to material differences between the actual future results, financial situation, development or performance of the company and the estimates given here. These factors include those discussed in Bayer’s public reports which are available on the Bayer website at www.bayer.com. The company assumes no liability whatsoever to update these forward-looking statements or to conform them to future events or developments.

Bayer AG is a holding company with operating subsidiaries worldwide. References to “Bayer” or “the company” herein may refer to one or more subsidiaries as context requires.