News Release

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Not intended for UK Media

FDA grants Orphan Drug Designation to BlueRock Therapeutics' investigational cell therapy OpCT-001

- Designation is for treating retinitis pigmentosa, one of the most common types of inherited retinal disorders characterized by the loss of photoreceptor cells
- OpCT-001 is an iPSC-derived investigational cell therapy being clinically tested to treat retinitis pigmentosa

Berlin, Germany, and Cambridge, MA, USA January 22, 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, OpCT-001, has received Orphan Drug Designation from the U.S. FDA for treating retinitis pigmentosa (RP).

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 and cone-rod dystrophy. Retinitis pigmentosa is one of the most common types of inherited retinal diseases characterized by the loss of rod and cone cells, known together as photoreceptor cells. OpCT-001 aims to restore vision in people living with RP by replacing lost cells in the retina with new functional cells.

"Receiving Orphan Drug Designation for retinitis pigmentosa is an important milestone for the OpCT-001 program," said Amit Rakhit, MD, MBA, BlueRock's Chief Medical Officer. "We believe that OpCT-001 has great promise as a potential therapeutic option for restoring vision in people living with retinitis pigmentosa and other primary photoreceptor diseases and look forward to continuing to work with the FDA on its clinical development."

Orphan Drug Designation is given to drugs and biologics for the prevention, diagnosis, or treatment of diseases or conditions affecting fewer than 200,000 persons in the U.S.[1]

“The FDA’s Orphan Drug Designation for OpCT-001 to treat retinitis pigmentosa underscores the importance of developing urgently needed innovative therapies for patients living with inherited retinal disorders,” said Christian Rommel, Executive Vice President and Global Head of Research and Development of the Pharmaceuticals Division at Bayer. “Together with BlueRock, we are excited to be advancing the first-ever clinical trial for an iPSC-derived cell therapy in primary photoreceptor diseases.”

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.

[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

About CLARICO
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 US 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 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 help people and the planet thrive by supporting efforts to master the major challenges presented by a growing and aging global population. Bayer is committed to driving sustainable development and generating a positive impact with its businesses. At the same time, the Group aims to increase its earning power and create value through innovation and growth. The Bayer brand stands for trust, reliability and quality throughout the world. In fiscal 2024, the Group employed around 93,000 people and had sales of 46.6 billion euros. R&D expenses amounted to 6.2 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 Opsis Therapeutics, the ability to advance therapies into, and successfully initiate, enroll 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.