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News Release

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BlueRock Therapeutics' investigational cell therapy bemdaneprocel for Parkinson's disease shows positive data at 24-months

- Bemdaneprocel is the most clinically advanced investigational cell therapy in the U.S. for treating individuals living with Parkinson's disease
- At 24 months, data from the Phase 1 exPDite trial continue to show a favorable safety profile in all 12 participants in the trial's high and low dose cohorts
- Secondary clinical endpoints continue to suggest positive trends from baseline through the duration of follow-up, with more encouraging trends in the high dose cohort than those in the low dose cohort
- Data will be presented at the International Congress of Parkinson's Disease and Movement Disorders in Philadelphia on September 28, 2024

Berlin, Germany, and Cambridge, MA, USA, September 27, 2024 – Bayer AG and BlueRock Therapeutics LP, a clinical stage cell therapy company and wholly owned independently operated subsidiary of Bayer AG today announced positive 24-month data from exPDite, a Phase 1 clinical trial of bemdaneprocel, an investigational cell therapy for the treatment of Parkinson's disease.

Bemdaneprocel is the most clinically advanced investigational cell therapy in the U.S. for treating patients living with Parkinson's disease. The exPDite trial, designed to assess the safety and tolerability of bemdaneprocel, is now complete and the 24-month data will be presented at the International Congress of Parkinson's Disease and Movement Disorders in Philadelphia on September 28, 2024.

"We are very excited to share the 24-month data from the exPDite trial which shows that bemdaneprocel could be a potentially meaningful treatment option for individuals living with Parkinson's disease," said Amit Rakhit, Chief Development and Medical Officer at

BlueRock Therapeutics. “The completion of this study marks an important milestone for bemdaneprocel and sets the stage for the next phase of clinical development.”

The safety profile at 24 months is consistent with earlier findings, demonstrating that bemdaneprocel continues to be well-tolerated by patients, with no adverse events reported related to bemdaneprocel. Transplanted cells continue to survive and engraft in the brain after discontinuing immunosuppression therapy at 12 months as outlined in the study’s protocol. In addition, secondary clinical endpoints related to motor symptoms continue to show positive trends from baseline through the duration of follow-up, with more encouraging trends in the high dose cohort than those in the low dose cohort. These were assessed by the MDS-Unified Parkinson’s Disease Rating Scale Part II and III (MDS-UPDRS Part II & III) and the Hauser PD Diary, tools used to assess Parkinson’s disease severity in motor symptoms. These consistent positive trends suggest that bemdaneprocel could potentially offer sustained benefit for movement impairments caused by the disease.

“There is considerable momentum in the concept of restoring dopamine inputs in the brain using transplanted cells, and the positive results from the exPDite trial leads the drive forward,” said Claire Henchcliffe, MD, chair of the UCI School of Medicine Department of Neurology at the University of California, Irvine and one of the study’s Principal Investigators. “The completed study demonstrates that the transplanted cells survive and there are early signs that bemdaneprocel can potentially help patients to better control their motor symptoms. These are exciting results that warrant further exploration in a next phase placebo-controlled study.”

In the high dose cohort, the 24-month measurement of the effects of bemdaneprocel on motor symptoms using MDS-UPDRS Part III measured in the “OFF”-medication state, showed a mean reduction of 21.9 points compared with baseline. The low dose cohort showed a mean decrease of 8.3 points.

Using the Hauser PD Diary, which categorizes patients as being in the “ON” state when their symptoms are well controlled and in the “OFF” state when they experience a worsening of their symptoms, the 7 participants in the high dose cohort showed a mean increase of 1.8 hours in time spent in the “Good ON” state without troublesome dyskinesias compared with baseline after 24 months. Time spent in the “OFF” state showed a mean decrease of 1.9 hours from baseline after 24 months. The 5 participants

in the low dose cohort showed a mean decrease of 0.8 hours in the “Good ON” state time compared with baseline and a corresponding mean increase of 0.4 hours in “OFF” state time.

In the high dose cohort, the 24-month measurement of the effects of bemdaneprocel on activities of daily living using MDS-UPDRS Part II measured a mean reduction of 3.4 points compared with baseline. The low dose cohort showed a mean increase of 2.0 points.

“The continued positive results of the exploratory clinical endpoints for bemdaneprocel after 24 months are encouraging and support our commitment in developing innovative therapies that can significantly improve patient lives,” said Christian Rommel Head of Research and Development at Bayer’s Pharmaceuticals Division. “The emerging data confirm the potential for Parkinson's patients.”

About bemdaneprocel (BRT-DA01) and the exPDite Phase 1 trial
Bemdaneprocel (BRT-DA01) is an investigational cell therapy designed to replace the dopamine producing neurons that are lost in Parkinson’s disease. These dopaminergic neuron precursors are derived from pluripotent stem cells that are human embryonic stem cells. In a surgical procedure, these neuron precursors are implanted into the brain of a person with Parkinson’s disease. When transplanted, they have the potential to reform neural networks that have been severely affected by Parkinson’s and to restore motor and non-motor function to patients. In May 2024, bemdaneprocel received a Regenerative Medicine Advanced Therapy (RMAT) designation from the FDA which enables expedited development review and development planning guidance from senior managers with the FDA’s Center for Biologics Evaluation and Research (CBER). Bemdaneprocel has not been approved for treatment of any disease or medical condition by any health authority.

exPDite is a multi-center, multi-site, open-label, non-randomized, non-controlled phase 1 study. Twelve (12) subjects diagnosed with Parkinson’s disease received surgical transplantation of 1 of 2 different dose levels of bemdaneprocel to the post-commissural putamen bilaterally, and administration of a 1-year immunosuppression regimen. Cohort A (5 subjects) received a dose of 0.9 million cells per putamen. Cohort B (7 subjects) received 2.7 million cells per putamen. Safety and tolerability were assessed at 12 months as the primary endpoint, along with evidence of cell survival and motor effects.

The feasibility of transplantation was also assessed. Assessments were repeated at 18 months with final assessments at 24 months.

The transplant surgeries were performed by Dr. Viviane Tabar, MD, Chair of the Department of Neurosurgery at Memorial Sloan Kettering (MSK) Cancer Center and Dr. Andres Lozano, M.D., Ph.D., F.R.C.S.C., F.R.S.C., F.C.A.H.S., Neurosurgeon and Senior Scientist, Krembil Brain Institute, University Health Network (UHN), Alan & Susan Hudson Cornerstone Chair in Neurosurgery, Toronto Western Hospital, University Health Network and Chairman of the Division of Neurosurgery at the University of Toronto (UoT). Participants were followed at clinical sites by Dr. Harini Sarva, M.D. at Weill Cornell Medicine, Dr. Claire Henchcliffe, M.D., D.Phil., F.A.A.N., F.A.N.A. at the University of California, Irvine, and Dr. Alfonso Fasano, M.D., PhD., Chair in Neuromodulation and Multi-Disciplinary Care at the University Health Network (UHN) and UoT.

Disclosure:

Memorial Sloan Kettering (MSK): Dr. Tabar has financial interests related to BlueRock. MSK has institutional financial interests related to BlueRock. Note the foregoing institutional disclosure language is included because the referenced study relates to MSK technology licensed to BlueRock.

University Health Network (UHN): UHN has institutional financial interests related to BlueRock.

More information about the Phase I trial is available at clinicaltrials.gov (NCT04802733).

About Parkinson's disease

Parkinson's disease is a progressive neurodegenerative disorder caused by the death of specific nerve cells in the brain, leading to decreased dopamine levels. At diagnosis, it is estimated that patients have already lost 50-80% of their dopaminergic neurons. The loss of these neurons leads to a progressive loss of motor function and symptoms such as tremors, muscle rigidity, and slowness of movement. Even with medication, the symptoms of Parkinson's disease can fluctuate during the course of the day. According to the Parkinson's Foundation, more than 10 million people worldwide suffer from Parkinson's disease, with approximately one million living in the United States. There is no cure, and

the effectiveness of current treatments decreases over time so that new therapies are needed.

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, ophthalmic, cardiovascular and immunological diseases. Two of our novel investigational cell therapies, bemdaneprocel (BRT-DA01) for the treatment Parkinson's disease and OpCT-001 for the treatment of primary photoreceptor disease are clinical stage programs. Bemdaneprocel has RMAT (Regenerative Medicine Advanced Therapy) and Fast Track designation from the US FDA (Food and Drug Administration); has completed Phase 1 clinical trial and is progressing to the next stage of clinical development. OpCT-001 has been cleared by the FDA to begin phase 1 clinical testing. 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 as a cornerstone of its newly formed Cell & Gene Therapy platform. 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 2023, the Group employed around 100,000 people and had sales of 47.6 billion euros. R&D expenses before special items amounted to 5.8 billion euros. For more information, go to www.bayer.com.

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