Bayer AG
Communications
51368 Leverkusen
Germany
Phone +49 214 30-1
www.bayer.com/en/media

News Release

Not intended for UK Media

BlueRock Therapeutics receives FDA Regenerative Medicine Advanced Therapy designation for Parkinson's disease cell therapy candidate bemdaneprocel

- Regenerative Medicine Advanced Therapy (RMAT) designation follows phase I clinical trial results demonstrating that bemdaneprocel is well tolerated with no major safety issues through 18 months
- Bemdaneprocel is the most clinically advanced investigational cell therapy in the U.S. for treating patients living with Parkinson's disease

Berlin, Germany and Cambridge, MA, USA, May 30, 2024 – Bayer AG and BlueRock Therapeutics LP, a clinical stage cell therapy company and wholly owned independently operated subsidiary of Bayer AG, announced today that BlueRock's investigational cell therapy bemdaneprocel for the treatment of Parkinson's disease has been granted Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA).

"We are excited about the positive data from the bemdaneprocel phase I clinical trial and believe it has great potential to help patients living with Parkinson's disease regain functions they have lost to the disease," said Seth Ettenberg, President and CEO of BlueRock Therapeutics. "Now with this RMAT designation in hand, we look forward to closely collaborating with the FDA to ready this program for phase II clinical studies."

Bemdaneprocel is the most clinically advanced investigational cell therapy in the U.S. for treating Parkinson's disease. Phase I clinical trial data announced in March demonstrated that at 18 months it was well tolerated with no major safety issues. In addition, an observed increase in the F-DOPA PET imaging signal after stopping immune suppression

therapy at 12 months, as defined in the study's protocol, demonstrates that the transplanted cells survive and engraft in the brain.

Managed by the FDA's Center for Biologics, Evaluation and Research (CBER), the RMAT program is dedicated to investigational regenerative medicines, including cell therapies, intended to treat, modify, reverse, or cure serious diseases. Investigational therapies that receive RMAT designation are eligible to receive expedited development review and development planning guidance from senior CBER managers. RMAT also creates a pathway for early discussions about potential surrogate endpoints and ways to support accelerated approval and satisfy post approval requirements.

"The RMAT designation for bemdaneprocel underscores the potential of this candidate to fundamentally change the way we think about Parkinson's disease care," said Christian Rommel, PhD, Head of Research and Development at Bayer's Pharmaceuticals Division. "We are driven by our commitment to deliver breakthrough innovation for patients and are proud and excited to see bemdaneprocel continuing to clear hurdles in the development process."

About bemdaneprocel (BRT-DA01) and the Phase I 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 restore motor and non-motor function to patients. Bemdaneprocel has not been approved for treatment of any disease or medical condition by any health authority.

This phase I study is a multi-center, multi-site, open-label, non-randomized, non-controlled study. Twelve (12) subjects diagnosed with Parkinson's disease received surgical transplantation of 1 of 2 different dose levels of bemdaneprocel cells 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 1 year 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. All assessments will continue over 2 years.

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 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.

About BlueRock Therapeutics LP

BlueRock Therapeutics LP is a clinical stage cell therapy company focused on creating cellular medicines to reverse devastating diseases. We are harnessing the power of cell therapy to create a pipeline of new medicines for patients suffering from neurological, cardiovascular, immunological, and ophthalmic diseases. Our lead clinical program, bemdaneprocel, (BRT-DA01) is in Phase I clinical trials for Parkinson's disease. We were 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.

BlueRock Therapeutics Media Contact:

Jeff Lockwood, phone +1 617.510.6997

Email: jlockwood@bluerocktx.com

Bayer Media Contact:

Dr. Nuria Aiguabella Font, phone +49 1732329691

Email: nuria.aiguabellafont@bayer.com

Bayer U.S. Media Contact:
Carolyn Nagle, phone +1 2014190337
Email: carolyn.nagle@bayer.com

Contact for investor inquiries:
Bayer Investor Relations Team, phone +49 214 30-72704
Email: ir@bayer.com
www.bayer.com/en/investors/ir-team

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naf (2024-0096e)

Forward-Looking Statements
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