BlueRock's Phase I study with bemdaneprocel in patients with Parkinson's disease meets primary endpoint

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- Investigational cellular therapy, bemdaneprocel (BRT-DA01), was well tolerated with no major safety issues in all 12 participants in low dose and high dose cohorts through one year.
- At one-year, exploratory clinical endpoints improved overall, with participants in the high dose cohort showing greater improvement.
- One year assessment of all participants demonstrated feasibility of transplantation, cell survival, and engraftment.
- Planning is underway for a Phase II study that is expected to begin enrolling participants in H1 (first half) 2024.

BERLIN and CAMBRIDGE, Mass., Aug. 28, 2023 /PRNewswire/ -- Bayer AG and BlueRock Therapeutics LP, a clinical stage cell therapy company and wholly owned independently operated subsidiary of Bayer AG, announced today details of the positive data from the Phase I clinical trial for bemdaneprocel (BRT-DA01), a stem cell derived investigational therapy for treating Parkinson's disease. The data were presented at the International Congress of Parkinson's Disease and Movement Disorders® in Copenhagen, Denmark.

The study met the primary objective of demonstrating safety and tolerability in all 12 participants in the study's low and high dose cohorts, with no serious adverse events (SAEs) reported related to bemdaneprocel through one year. There were two SAEs reported that were unrelated to bemdaneprocel, one seizure attributed to the surgical procedure and one COVID case. Both resolved without sequelae. In addition, 18F-DOPA PET imaging scans demonstrated evidence of cell survival and engraftment in both low and high dose cohorts. 18F-DOPA PET imaging is a neuroradiological technique used to visualize and assess dopaminergic activity in Parkinson's disease.

Secondary exploratory clinical endpoints improved in both cohorts, with participants in the high dose cohort showing greater improvement, as assessed by the MDS-Unified Parkinson's Disease Rating Scale Part III (MDS-UPDRS Part III) and the Hauser Diary, which are tools used to assess Parkinson's disease severity in motor symptoms.

"The data from this Phase I open label study are extremely encouraging," 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. "While this is a small open label study, meeting the study's primary objective for safety and tolerability along with initial improvements seen in clinical outcomes represents a great step forward. The hope now is that these trends continue and translate into meaningful benefit for people with Parkinson's disease in controlled clinical trials."

Using the Hauser 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, participants in the high dose cohort showed an improvement of 2.16 hours in time spent in the "ON" state without troubling dyskinesia compared with baseline after one year. Time spent in the "OFF" state showed a corresponding decrease of 1.91 hours after one year. Participants in the low dose cohort showed an improvement of 0.72 hours in the "ON" state without troubling dyskinesia time compared with baseline and a corresponding decrease of 0.75 hours in "OFF" state time.

In the high dose cohort, a one-year measurement of the effects of bemdaneprocel using MDS-UPDRS Part III measured in the "OFF"-medication state, showed a reduction of 13.0 points compared with baseline. The low dose cohort showed a reduction of 7.6 points.

"The need for new therapies to help patients struggling with Parkinson's disease is clear," said Ahmed Enayetallah, Senior Vice President and Head of Development, BlueRock Therapeutics. "We are excited to be sharing the results of this Phase I and look forward to advancing bemdaneprocel to the next stage of clinical testing."

Based on these results, planning is underway for a Phase II study that is expected to begin enrolling patients in H1 (first half) 2024.

"The standard of care for millions of people living with Parkinson's disease has only marginally improved in the past decades, and the existing unmet medical need will only become higher due to the growing aging population," said Christian Rommel, Member of the Executive Committee of Bayer's Pharmaceuticals Division and Head of Research and Development. "The positive outcome of this Phase I clinical trial is a clear step forward, and it brings us closer to delivering new treatment options to patients."

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 (PSC) 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.

This phase I study is a multi-center, multi-site, open-label, non-randomized, non-controlled study. Twelve (12) subjects 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, along with evidence of cell survival and motor effects. The feasibility of transplantation was also assessed. 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

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