

News Release

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BlueRock Therapeutics phase I clinical trial for Parkinson's disease continues to show positive trends at 18 months

- Investigational cell therapy bemdaneprocel continues to be well tolerated with no major safety issues in all 12 participants in low and high dose cohorts through 18 months
- After stopping the 12-month immune suppression regimen, assessment of 18-month data shows evidence of sustained cell engraftment and increased F-DOPA signal
- Exploratory clinical endpoints continued to improve from 12 to 18 months, with participants in the high dose cohort showing greater improvement compared to the low dose cohort
- A phase II study is expected to begin enrolling participants later this year

Berlin, Germany and Cambridge, MA USA, March 6, 2024 – 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 18-month data from the phase I clinical trial for bemdaneprocel, an investigational allogeneic stem cell derived cell therapy for treating Parkinson's disease. The data were presented at the Alzheimer's and Parkinson's Diseases Conference in Lisbon, Portugal.

The data demonstrate that at 18 months bemdaneprocel continues to be well tolerated with no major safety issues, transplanted cells survive and engraft in the brain and F-DOPA signal continues to increase after stopping immune suppression therapy at 12 months as outlined in the study's protocol.

In addition, exploratory clinical endpoints improved compared to baseline assessments in both cohorts, with participants in the high dose cohort showing greater improvement than those in the low dose cohort. These were 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.

"It's exciting that bemdaneprocel met safety and tolerability criteria at 12 months, and now the 18-month results suggest that these allogeneic cells survive and have potentially positive effects even after discontinuation of immunosuppressants," 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. "We should not overinterpret results of a phase I study, but this is a promising step that deserves to be followed up with further studies."

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 a mean increase of 2.7 hours in time spent in the "Good ON" state time compared with baseline after 18 months. Time spent in the "OFF" state showed a mean decrease of 2.7 hours after 18 months. Participants in the low dose cohort showed a mean improvement of 0.2 hours in the "Good ON" state time compared with baseline and a corresponding mean decrease of 0.8 hours in "OFF" state time.

In the high dose cohort, an 18-month measurement of the effects of bemdaneprocel using MDS-UPDRS Part III measured in the "OFF"-medication state, showed a mean reduction of 23 points compared with baseline. The low dose cohort showed a mild improvement, with a mean decrease of 8.6 points.

"We are excited to see the continued positive trends in the data from bemdaneprocel's phase I trial at 18 months," said Ahmed Enayetallah, Senior Vice President and Head of Development at BlueRock Therapeutics. "While it is still early days and there is more work to do, we look forward to further investigating bemdaneprocel in clinical studies."

A phase II study for further clinical testing of bemdaneprocel is planned to begin enrolling patients later this year.

"We are on the leading edge in the research for new treatment options for Parkinson's disease as bemdaneprocel, the most clinically advanced pluripotent stem derived cell therapy candidate to date for this disease, continues to show positive trends," said

Christian Rommel, Member of the Executive Committee of Bayer's Pharmaceuticals Division and Head of Research and Development. "There are good reasons to be optimistic about these early data, and we are excited to move to phase II later this year."

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](https://clinicaltrials.gov/ct2/show/study/NCT04802733) ([NCT04802733](https://clinicaltrials.gov/ct2/show/study/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

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