



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

News Release

Not intended for US and UK Media

Bayer first company to advance cell therapy as well as gene therapy against Parkinson's disease

- First investigational allogeneic pluripotent stem cell derived therapy pivotal Phase III clinical trial exPDite-2 with bemdaneprocel started
 - Phase II gene therapy trial REGENERATE-PD with AB-1005 initiated randomizing participants in United Kingdom and Poland, with Germany following shortly and ongoing in the USA
 - Parkinson's disease second most common and fastest growing neurodegenerative disease affecting more than 10 million people worldwide
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Berlin, September 22, 2025 – Bayer announced today progress for two potential therapies against Parkinson's disease (PD). A first participant received randomized treatment in the pivotal Phase III clinical trial, exPDite-2, of bemdaneprocel, an investigational cell therapy for PD. At the same time, first European participants have been randomized in REGENERATE-PD, a Phase II clinical trial of AB-1005, an investigational gene therapy. Both therapies are focused on treating moderate-stage PD and are being developed in collaboration with Bayer's wholly owned, independently operated subsidiary companies, BlueRock Therapeutics LP (bemdaneprocel) and AskBio Inc. (AB-1005) respectively.

"Our dual approach in addressing Parkinson's disease through both cell and gene therapies exemplifies our strategic vision and maximizes our chances of offering renewed hope for Parkinson's patients who have been waiting for new therapies for too long," said Christian Rommel, Global Head of Research and Development of Bayer's Pharmaceutical Division. "We are energized by being at the forefront of the development of disease modifying therapies that have the potential to make a meaningful impact on Parkinson's patients lives."

Bayer is setting a global benchmark in the development, manufacturing, and commercialization of cell and gene therapies based on the establishment of a comprehensive end-to-end approach that covers the entire spectrum from research to production and includes state-of-the-art production facilities worldwide.

“Parkinson’s disease is characterized by the loss of dopamine producing cells in the brain resulting in motor impairments,” said Gabi Belfort, MD, PhD, Senior Vice President and Bemdaneprocel Product Lead at BlueRock Therapeutics. “Bemdaneprocel aims to replace these lost cells with new dopamine producing neurons and we are excited that the pivotal Phase III exPDite-2 clinical trial to explore this approach is now underway.”

“We believe there is a significant need for neurorestorative therapies in Parkinson’s, and AskBio is committed to exploring the potential of investigational gene therapy AB-1005 in the treatment of this severe, progressive chronic disease,” said Adrian Kells, PhD, Senior Vice President, Integrated Product Team Lead, Parkinsons and MSA, at AskBio. “We are excited to share the news of the advancement of our REGENERATE-PD clinical trial in Europe, which we believe is an important update for patients and the medical community.”

Bemdaneprocel and AB-1005 are investigational therapies which have not been approved by any regulatory authority, and their efficacy and safety have not been established or fully evaluated.

About Parkinson’s Disease

Parkinson’s disease (PD) is a progressive neurodegenerative disease. It has a significant impact on a person’s daily life. In PD, the death of dopamine producing nerve cells in the brain leads to the continuous loss of motor function. Symptoms include tremors, muscle rigidity, and slowness of movement. Additionally, people with PD experience non-motor symptoms, including fatigue and lack of energy, cognitive issues, and depression. Symptoms typically intensify over time and make everyday tasks demanding. The prevalence of PD has doubled over the past 25 years. Today, more than 10 million people worldwide are estimated to be living with PD. This makes it the world’s second most prevalent neurodegenerative disease. It is also the most frequent movement disorder. At present there is no cure, and current treatment options are inadequate and lack the holistic management of symptoms so there is an urgent need for new therapies.

About Bemdaneprocel (BRT-DA01)

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 human embryonic pluripotent stem cells that continue developing into mature dopamine neurons after implantation. 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 re-form neural networks that have been severely affected by Parkinson's disease and to potentially restore motor and non-motor function to patients. In 2021 bemdaneprocel received Fast Track Designation and in 2024 a Regenerative Medicine Advanced Therapy (RMAT) designation from the FDA. Data from the Phase I trial's 12 participants presented at the 2024 International Congress of Parkinson's Disease and Movement Disorders (MDS) demonstrated good tolerability, with no serious adverse events related to drug product at 24 months post-surgery. Further, encouraging trends were observed in secondary endpoints related to motor impairments at 24 months post-surgery. These participants continue in the long term Continued Evaluation Study. Bemdaneprocel has not been approved for treatment of any disease or medical condition by any health authority.

About exPDite-2

exPDite-2 is the first Phase III pivotal clinical trial for an investigational allogeneic pluripotent stem cell derived therapy to treat Parkinson's disease. In a Phase I study with 12 participants, bemdaneprocel was well tolerated, with no serious adverse events related to drug product at 24 months post-surgery. In addition, encouraging trends were observed in secondary endpoints related to motor impairments at 24 months post-surgery. Building on these results, exPDite-2 is a multicenter, double-blind trial that will assess the efficacy, safety and overall impact of bemdaneprocel compared to a sham surgery control. The trial is designed to enroll approximately 102 participants with Parkinson's disease. The primary endpoint of the study is change from baseline to week 78 in PD diary measure of ON-time without troublesome dyskinesia, adjusted for a 16-hour waking day. In addition, the trial will incorporate secondary endpoints designed to assess objective measures of movement, non-motor symptoms, safety and tolerability, and instruments that capture activities of daily living and quality of life. For more information about the exPDite-2 clinical trial, visit clinicaltrials.gov (NCT06944522), or visit bluerocktx.com.

Depending upon the outcome, the results from this trial are intended to be part of a data package to support regulatory submissions for marketing authorization.

About AB-1005

AB-1005 is an investigational gene therapy based on adeno-associated viral vector serotype 2 (AAV2) containing the human glial cell line-derived neurotrophic factor (GDNF) transgene, which allows for stable and continuous expression of GDNF in localized regions of the brain after direct neurosurgical injection with MRI-monitored convection enhanced delivery. In nonclinical studies, GDNF has been shown to promote the survival and morphological differentiation of dopaminergic neurons. Recombinant GDNF has long been evaluated as a potential treatment for diseases, such as PD, marked by progressive degeneration of midbrain dopaminergic neurons. Through a combination of an investigational gene therapy and innovative neurosurgical delivery approach, one can now test the GDNF hypothesis in PD by getting this neurotrophic factor to these degenerating nigrostriatal neurons in a potentially more clinically relevant fashion.

About REGENERATE-PD

REGENERATE-PD is a Phase II, randomized, double-blind, surgery controlled trial of the efficacy and safety of intraputaminaly administered investigational gene therapy AB-1005 in the treatment of adults (45–75 years) with moderate-stage Parkinson's disease. The trial will include an estimated 87 participants with trial sites located in Germany, Poland, the United Kingdom, and the United States. For more information about the REGENERATE-PD clinical trial, visit [clinicaltrials.gov](https://clinicaltrials.gov/ct2/show/study/NCT06285643) (NCT06285643), or visit askbio.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.

Contact for media inquiries:

Dr. Imke Meyer, phone +49 214 6000 1275

Email: imke.meyer@bayer.com

Contact for investor inquiries:

Bayer Investor Relations Team, phone +49 214 30-72704

Email: ir@bayer.com

www.bayer.com/de/investoren/ir-team

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ime (2025-0166E)

Forward-Looking Statements

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