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

News Release

Bayer to advance two first-of-its-kind cell and gene therapies for Parkinson's disease

- Parkinson's disease is most common neurodegenerative movement disorder
- It impacts more than 10 million people worldwide
- No function-restoring therapy is currently available
- Bayer is pursuing a two-pronged approach to deliver transformative therapies with one cell and one gene therapy candidate in clinical trials

Berlin, Germany, June 8, 2021 – Bayer AG announced today that BlueRock Therapeutics (BlueRock), a clinical stage biopharmaceutical company and wholly-owned subsidiary of Bayer AG, successfully administered the first dose of its pluripotent stem cell-derived dopaminergic neurons, named DA01, to a Parkinson's disease patient in their open-label Phase 1 clinical study. In parallel a gene therapy program also targeted at providing advanced therapies for Parkinson's disease is driven forward by Bayer's wholly-owned clinical-stage adeno-associated virus (AAV) gene therapy company Asklepios BioPharmaceutical, Inc. (AskBio). This program is currently recruiting and evaluating patients in an ongoing Phase 1b clinical study.

"The potential of BlueRock and AskBio's clinical candidates to treat Parkinson's disease could be immense," said Wolfram Carius, Head of Cell and Gene Therapy at Bayer. "For the first time, it might be possible to stop and reverse this degenerative disease and truly help patients with their high unmet medical need. The start of clinical trials represents the beginning towards a truly breakthrough treatment option to dramatically improve the lives of patients."

Parkinson's disease is the most common neurodegenerative movement disorder, impacting more than 10 million people worldwide. It is caused by nerve cell damage in the brain, leading to decreased levels of dopamine (a neurotransmitter involved in processes

such as memory or movement). The disease often starts with a tremor in one hand. Other symptoms include rigidity, cramping and dyskinesias (involuntary, erratic, writhing movements of the face, arms, legs or trunk). Dopamine substitutes, such as levodopa, are commonly used to mitigate the symptoms of the disease, but their effect diminishes as the disease progresses and there is currently no disease-modifying treatment available.
By targeting the disease at its root cause, cell and gene therapies aim to go beyond symptomatic treatments.

Using authentic dopaminergic neurons, BlueRock aims to re-innervate the affected regions of the human brain and reverse the degenerative process, potentially restoring motor function. BlueRock's clinical trial will enroll ten patients at sites in the United States of America (US) and Canada. In this study, patients undergo surgical transplantation of the dopamine-producing cells into the putamen, a deep brain structure affected by Parkinson's disease. The primary objective of the Phase 1 study (NCT04802733) is to assess the safety and tolerability of DA01 cell transplantation at one-year post-transplant. The secondary objectives of the study are to assess the evidence of transplanted cell survival and motor effects at one- and two-years post-transplant, to evaluate continued safety and tolerability at two years, and to assess feasibility of transplantation.

AskBio's approach consists of an AAV that delivers human glial cell line-derived neurotrophic factor (GDNF) gene to the neurons within the putamen, resulting in expression and secretion of GDNF protein in brain regions impacted by Parkinson's disease. Long-term experiments using AAV-GDNF showed that sustained expression of GDNF can promote regeneration of midbrain neurons and significant motor recovery in rodents and non-human primate models. AskBio's clinical study is currently recruiting and evaluating patients in Phase 1b in the US to assess safety and preliminary efficacy.
A total of 10 patients have been enrolled since the start of the Phase 1b study (NCT04167540) in August 2020.

About Bayer
Bayer is a global enterprise with core competencies in the life science fields of health care and nutrition. Its products and services are designed to help people and planet thrive by supporting efforts to master the major challenges presented by a growing and aging global population. Bayer is committed to drive sustainable development and generate 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 2020, the Group employed around 100,000 people and had sales of 41.4 billion euros. R&D expenses before special items amounted to 4.9 billion euros. For more information, go to www.bayer.com.

About AskBio

Asklepios BioPharmaceutical, Inc. (AskBio), a wholly owned and independently operated subsidiary of Bayer AG acquired in 2020, is a fully integrated AAV gene therapy company dedicated to developing life-saving medicines that cure genetic diseases. The company maintains a portfolio of clinical programs across a range of neuromuscular, central nervous system, cardiovascular and metabolic disease indications with a clinical-stage pipeline that includes therapeutics for Pompe disease, Parkinson's disease and congestive heart failure, as well as out-licensed clinical indications for hemophilia and Duchenne muscular dystrophy. AskBio's gene therapy platform includes Pro10™, an industry-leading proprietary cell line manufacturing process, and an extensive AAV capsid and promoter library. With global headquarters in Research Triangle Park, North Carolina, and European headquarters in Edinburgh, UK, the company has generated hundreds of proprietary third-generation AAV capsids and promoters, several of which have entered clinical testing. Founded in 2001 and an early innovator in the gene therapy field, the company holds more than 500 patents in areas such as AAV production and chimeric and self-complementary capsids. Learn more at www.askbio.com or follow on LinkedIn.

About BlueRock Therapeutics

BlueRock Therapeutics is an engineered cell therapy company with a mission to develop regenerative medicines for intractable diseases. The company's cell+gene platform enables the creation, manufacture, and delivery of authentic cell therapies with engineered functionality by simultaneously harnessing pluripotent cell biology and genome editing. This enables an approach where, in theory, any cell in the body can be manufactured and any gene in the genome can be engineered for therapeutic purposes. The platform is broadly applicable, but the company is focused today in neurology, cardiology, and immunology. In August 2019, the company was acquired by Bayer AG, for an enterprise value of $1B in upfront and milestone payments. For BlueRock this marks the next step in the journey to prove degenerative disease is reversible, and to bring its revolutionary new medicines to the patients who desperately need them. For more information, visit bluerocktx.com.

Contact for media inquiries:
Nuria Aiguabella Font, phone +49 30 468-193 131
Email: nuria.aiguabellafont@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

Find more information at https://pharma.bayer.com/
Follow us on Facebook: http://www.facebook.com/pharma.bayer
Follow us on Twitter: @BayerPharma

naf (2021-0113E)

Forward-Looking Statements
This release may contain forward-looking statements based on current assumptions and forecasts made by Bayer management. Various known and unknown risks, uncertainties and other factors could lead to material differences between the actual future results, financial situation, development or performance of the company and the estimates given here. These factors include those discussed in Bayer's public reports which are available on the Bayer website at www.bayer.com. The company assumes no liability whatsoever to update these forward-looking statements or to conform them to future events or developments.