



BlueRock Therapeutics in Collaboration with Memorial Sloan Kettering Cancer Center Receives IND Clearance for DA01 in Parkinson's Disease

Cambridge, MA, 01.07.21 – BlueRock Therapeutics, a preclinical stage biopharmaceutical company and wholly-owned subsidiary of Bayer AG, in collaboration with Memorial Sloan Kettering Cancer Center (MSK), announced today that the U.S. Food and Drug Administration (FDA) has cleared their Investigational New Drug (IND) application to proceed with a Phase 1 (Ph1) study in patients with advanced Parkinson's disease (PD). This is the first trial in the United States to study pluripotent stem cell-derived dopaminergic neurons in patients with Parkinson's disease. Under the IND, BlueRock and MSK will execute a Ph1 clinical trial to evaluate the safety, tolerability and preliminary efficacy of DA01 in patients with PD

"This is a big step for the stem cell field – to finally test a truly "off-the-shelf" dopamine neuron product in human PD patients," said Lorenz Studer, MD, scientific co-founder of BlueRock and Director, Center for Stem Cell Biology at MSK. "We are also grateful for the visionary support by NYSTEM, the NY state-sponsored stem cell program that supported the earlier stages of this project."

"This trial is the culmination of a decade of arduous collaborative work that is based on very rigorous science. It is an important milestone on the road towards regenerative brain repair," said Viviane Tabar, MD, founding investigator of BlueRock and Chair of MSK's Department of Neurosurgery. "It is a real privilege and very exciting to be able to participate in both the bench science and the actual surgical intervention, here at MSK. Our collaborators at Weill Cornell Neurology will also be an integral part of the trial."

"Today, there is no disease-modifying treatment for Parkinson's. Through this trial and those to follow, we hope to change that," stated Emile Nuwaysir, Ph.D., President and Chief Executive Officer of BlueRock. "Our therapy is intended to replace the midbrain dopaminergic neurons lost in the degenerative condition to rebuild the neural circuit, and thereby restore motor control to Parkinson's patients. This could shift the treatment paradigm for millions of PD patients, as well as demonstrate for the first time that

degenerative disease is, in principle, reversible. We believe this would represent an enormous step for the PD community worldwide, and for medicine.”

The trial plans to enroll ten patients starting with a first clinical site at Weill Cornell Medicine in the initial open-label study. The primary objective of the Ph1 study 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.

About Parkinson’s Disease

Parkinson’s disease is a progressive neurodegenerative disorder caused by nerve cell damage in the brain, leading to decreased dopamine levels. The worsening of motor and non-motor symptoms is caused by the loss of dopamine-producing neurons. At diagnosis, it is estimated that patients have already lost 60-80% of their dopaminergic neurons. Parkinson’s disease often starts with a tremor in one hand. Other symptoms are rigidity, cramping and dyskinesias. Parkinson’s disease is the second most common neurodegenerative disorder, impacting more than 7.5 million people, including 1.3 million people in North America.

About BlueRock Therapeutics

BlueRock Therapeutics is a leading engineered cell therapy company with a mission to develop regenerative medicines for intractable diseases. BlueRock Therapeutics’ *cell+gene* platform harnesses the power of cells to create new medicines for neurology, cardiology and immunology indications. BlueRock Therapeutics’ cell differentiation technology recapitulates the cell’s developmental biology to produce authentic cell therapies, which are further engineered for additional function. Utilizing these cell therapies to replace damaged or degenerated tissue brings the potential to restore or regenerate lost function. BlueRock Therapeutics was founded in 2016 by Versant Ventures and Bayer AG and capitalized with one of the largest-ever Series A financings in biotech history by Bayer AG (through its Leaps by Bayer unit) and Versant Ventures. The company was fully acquired by Bayer in 2019. BlueRock Therapeutics’ culture is defined by scientific innovation, the highest ethical standards and an urgency to bring transformative treatments to all who would benefit. For more information, visit bluerocktx.com.

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 benefit people by supporting efforts to overcome the major challenges presented by a growing and aging global population. At the same time, the Group aims to increase its earning power and create value through innovation and growth. Bayer is committed to the principles of sustainable development, and the Bayer brand stands for trust, reliability and quality throughout the world. In fiscal 2019, the Group employed around 104,000 people and had sales of 43.5 billion euros. Capital expenditures amounted to 2.9 billion euros, R&D expenses to 5.3 billion euros. For more information, visit bayer.com.

Disclosures

Dr. Studer has intellectual property rights and interests and financial interests related to BlueRock. Dr. Tabar has financial interests related to BlueRock. Researchers at Memorial Sloan Kettering Cancer Center, including Dr. Studer, developed stem cell-derived dopaminergic neurons for the treatment of neurodegenerative diseases, and MSK licensed this intellectual property to BlueRock. MSK has institutional financial interests related to this intellectual property and BlueRock.

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Forward-Looking Statements

Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may be identified by the use of forward-looking words such as "anticipate," "believe," "forecast," "estimate" and "intend," among others. These forward-looking statements are based on BlueRock's current expectations and actual results could differ materially. There are a number of factors that could cause actual events to differ materially from those indicated by such forward-looking statements. These factors include, but are not limited to, the timing of our clinical trial for DA01; our results regarding the safety, tolerance and efficacy of DA01 cell transplantation for patients with Parkinson's disease; and ongoing FDA and other regulatory requirements regarding the development of DA01. As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. Except as expressly required by law, BlueRock does not undertake an obligation to update or revise any forward-looking statement. All of the Company's forward-looking statements are expressly qualified by all such risk factors and other cautionary statements. The information set forth herein speaks only as of the date hereof.