AskBio Acquires BrainVectis to Expand its Clinical Pipeline for Neurodegenerative Diseases

BrainVectis adds Huntington's disease gene therapy candidate to AskBio's program

Research Triangle Park, N.C. and Paris, France – April 22, 2020 – Asklepios BioPharmaceutical, Inc. (AskBio), a leading, clinical-stage adeno-associated virus (AAV) gene therapy company, today announced that it has acquired BrainVectis, a Paris-based gene therapy company and French National Institute for Health and Medical Research (INSERM) spin-out with expertise and an intellectual property estate for the treatment of neurodegenerative disorders. BrainVectis is particularly focused on therapies that restore brain cholesterol metabolism as a treatment for Huntington's disease and other disorders. Financial terms of the acquisition were not disclosed.

Commenting on the transaction, Sheila Mikhail, CEO and co-founder of AskBio, said, "Our acquisition of BrainVectis offers us an extraordinary opportunity to work together to potentially transform patients' lives. Huntington's disease is a devastating and irreversible disease for which no effective treatments exist. BrainVectis has world-leading knowledge of Huntington's and other neurodegenerative diseases and gives us access to a pipeline of candidates for these indications. We're honored to have them become part of AskBio."

BrainVectis was founded by award-winning physician and gene therapy pioneer Nathalie Cartier-Lacave, M.D., with early funding and support from INSERM, Sorbonne University and the French Alternative Energies and Atomic Energy Commission (CEA). Dr. Cartier-Lacave and her team are working to develop gene therapies to increase expression of the CYP46A1 enzyme in the brain. This enzyme is linked to a number of diseases where brain cholesterol metabolism dysfunction is implicated in the pathology of neurodegeneration.

The company's lead gene therapy candidate, BV-CYP01, has shown proof-of-concept in various animal models of Huntington's disease and received Orphan Drug Designation (ODD) from the European Commission in April 2019.

"We have seen promising preclinical results with BV-CYP01, and combining our program with gene therapy leader AskBio is ideal as we progress toward clinical studies," said Dr. Cartier-Lacave. "With its unmatched technology platform, AskBio has the resources to accelerate the development of our gene therapies for patients who desperately need treatment options."

AskBio will leverage its proprietary capsid and synthetic promotor design technologies and manufacturing technology and capacity to advance the development of this and other BrainVectis programs.

BrainVectis will operate as a wholly owned subsidiary of AskBio and maintain its office in Paris, France.

About Dr. Nathalie Cartier-Lacave
Nathalie Cartier-Lacave, M.D., is the founder of BrainVectis and director of research at INSERM, where she leads the Cell and Gene Therapy for Neurodegenerative Diseases group at Paris Brain Institute, a prestigious multidisciplinary neurology center located at Pitié-Salpêtrière Hospital. For the

last decade, she has been investigating the link between brain cholesterol and neurodegenerative diseases. Her research led to the first clinical trials using a lentiviral vector for a gene therapy program against adrenoleukodystrophy, and she has progressed two gene therapy products into clinical studies for pediatric leukodystrophies. Dr. Cartier-Lacave is the past president of the European Society of Gene & Cell Therapy (ESGCT) and won its Outstanding Achievement award in 2019. She also received the Grand Prize from the Foundation for Medical Research, which honors an internationally renowned researcher for their exceptional contribution to the advancement of scientific knowledge in the medical field, and Guy Lazorthes Prize from the French Academy of Sciences in 2019.

About BrainVectis
Founded in 2015 as a spinoff from the French National Institute for Health and Medical Research (INSERM), BrainVectis develops gene therapy treatments for neurodegenerative diseases by targeting the cholesterol pathway in the brain to restore cholesterol metabolism. The company is currently studying treatments for Huntington's disease and Alzheimer's disease using the adeno-associated virus (AAV) to transfer the gene for the CYP46A1 enzyme into the human brain. BrainVectis has secured the intellectual property for both indications from INSERM and has filed new patent applications for other degenerative conditions. To learn more, please visit http://www.brainvectis.com/.

About AskBio
Founded in 2001, Asklepios BioPharmaceutical, Inc. (AskBio) is a privately held, clinical-stage gene therapy company dedicated to improving the lives of children and adults with genetic disorders. AskBio's gene therapy platform includes an industry-leading proprietary cell line manufacturing process called Pro10™ 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. An early innovator in the space, the company holds more than 500 patents in areas such as AAV production and chimeric and self-complementary capsids. AskBio has a portfolio of clinical programs across a range of neurodegenerative and neuromuscular indications that includes therapeutics for Pompe disease, limb-girdle muscular dystrophy 2i/R9 and congestive heart failure, as well as out-licensed clinical indications for hemophilia (Chatham Therapeutics acquired by Takeda) and Duchenne muscular dystrophy (Bamboo Therapeutics acquired by Pfizer). Learn more at https://www.askbio.com or follow us on LinkedIn.

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