



**BrainVectis** develops gene therapy strategies to treat neurodegenerative diseases. Huntington's disease is our most advanced program based on the injection of an AAV vector genetically modified to transfer the gene for the enzyme CYP46A1 into the human brain.

Cholesterol is an essential component of the neuronal cell membrane that enables the formation of synapses and synaptic transmission. Cholesterol contributes to all the basic processes of motility and cognition such as learning and memory. The metabolism of cerebral cholesterol is independent of the metabolism of peripheral cholesterol. Cerebral cholesterol is produced locally and its level is finely regulated. Any excess is potentially toxic for neurons and has to be eliminated. This is the role of CYP46A1 an enzyme that converts cholesterol to 24-OH-cholesterol able to cross the brain blood barrier for peripheral elimination.

Numerous animal study and clinical trial data have shown the role of disturbances in cholesterol metabolism in neurodegenerative diseases, particularly in **Huntington's disease (HD)** and **Alzheimer's disease (AD)** for which there is currently no effective treatment. In those two diseases, changes in cholesterol content are observed in the brain areas affected and are associated with a decrease in CYP46A1 activity and in the efflux of excess cholesterol from the brain.

We have shown, in several animal models of these two diseases and also in **spinocerebellar ataxias**, that supplying CYP46A1 to the brain using gene therapy enables not only prevention but also regression of the lesions. Our first product BV-CYP01 is based on an AAV vector, a non-toxic virus and potent therapeutic vector currently used in numerous clinical trials in man.

Our goal is to bring BV-CYP01 into the clinic early in 2020 in Huntington's disease.

**Intellectual property:** Brainvectis has a worldwide exclusive licence on two patents that protect our treatments in HD and AD. These patents are granted in the United States and in Europe. We file patent applications for new CNS conditions for which we got proof of concept in specific animal models.

**Team and partners:** 5 employees in 2017 growing to 10 in 2018. Jérôme Becquart is the CEO of Brainvectis that is scientifically coordinated by Nathalie Cartier. Preclinical research is headed by Sandro Alves. A scientific and medical committee has been set up with experts in HD, AD, gene therapy and in drug development.

Brainvectis laboratories are located at ICM (Pitié Salpêtrière Hospital, Paris). We have set up collaborations with CEA/MIRCen (primate studies), St-Antoine Hospital (analytics), Oniris and Nantes university (regulatory studies and biomanufacturing). Brainvectis has got the support from Paris Biotech Santé and Idfinnov.

**Financing:** Brainvectis raised 2 M€ from private financing and obtained public aids from ANR and BPI.

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