Institute of Animal Physiology and Genetics CAS

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Institute of Animal Physiology and Genetics CAS
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CLINICAL TRIALS OF A NEW TREATMENT FOR HUNTINGTON'S DISEASE MAY BEGIN IN EUROPE THANKS TO MINIPIGS FROM LIBĚCHOV

The Liběchov minipigs from the PIGMOD Center of the Institute of Animal Physiology and Genetics CAS were used in the pre-clinical testing of a new therapy for Huntington's disease, a serious human disorder affecting the brain. The outcoming promising results have paved the way for the therapy to enter the clinical trial phase, bringing the actual treatment options for this disease closer to patients in Europe.

Scientists from the PIGMOD Center of the Institute of Animal Physiology and Genetics CAS have been studying Huntington's disease for years. This very serious human disorder affecting the brain is caused by a mutation in the gene encoding the huntingtin protein. *"The tested therapy aims to reduce the amount of huntingtin directly in the animal brain using a short interfering ribonucleic acid molecule (siRNA) that directly targets the huntingtin sequence,"* says the Head of PIGMOD Center Jan Motlík, explaining the new approach. A viral vector carrying a short siRNA molecule is injected directly into the brain using a disposable surgical syringe under general anaesthesia. *"The data collected afterwards showed a decrease in the amount of huntingtin after both 6 and 12 months in the most important brain regions of the transgenic minipigs as well as in their basal ganglia and motor cortex,"* says the co-author of the publication Zdeňka Ellederová, describing the specific test results.

"Liběchov minipigs will help people, but they do not suffer from the disease themselves"

The Liběchov minipigs represent an absolutely unique model for the research of Huntington's disease. *"We created the minipigs as genetic models that carry mutant human or pig huntingtin, even though they are clinically healthy without any symptoms of Huntington's disease, a condition fatal and having a severe course in humans. That is why they serve as an ideal model for testing mutant huntingtin production suppression without suffering from the symptoms of Huntington's disease,"* says Zdeňka Ellederová,

describing the role of the transgenic pig model. On the basis of these promising pre-clinical test results, both European and U.S. regulatory agencies (FDA and EMA) have given their approval to start clinical testing in patients. *"Since in Autumn 2020 the FDA Safety Board positively evaluated the clinical study in the USA, clinical testing of this viral vector will be extended to patient in Europe in 2021,"* says Jan Motlík of the impact of the study.

The pre-clinical studies were conducted by scientists from the PIGMOD Center of the Institute of Animal Physiology and Genetics CAS in collaboration with neurologists from the Na Homolce Hospital, St. Anne's University Hospital and Veterinary University in Brno, all under the guidance of the Dutch company uniQure. In April, the results were published by the prestigious journal *Science Translational Medicine*.

Further information:

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Filming options at the minipig breeding facilities and in the operating rooms of the Institute of Animal Physiology and Genetics CAS in Liběchov.

Photos:

Fig. 1. Liběchov minipigs – an ideal model for the study of Huntington's disease (Photo: IAPG archive)

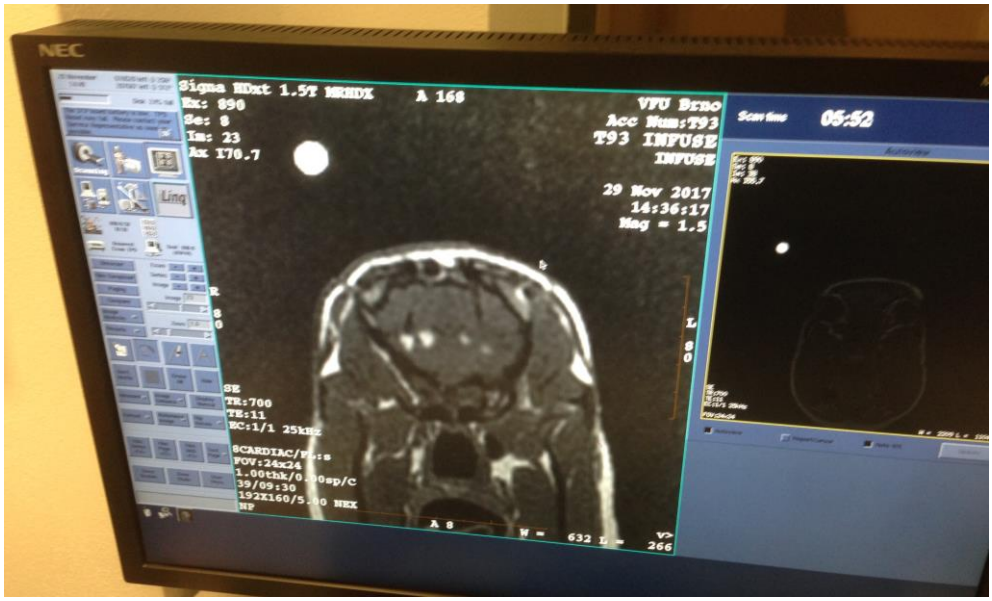

Fig. 2: MRI image with a contrast agent to verify the exact injection site for vector administration (Photo: Z. Ellederová)