FEDERATION OF EUROPEAN NEUROSCIENCE SOCIETIES
11th FENS Forum of Neuroscience
7-11 July 2018 – Berlin, Germany
https://forum2018.fens.org/

PRESS RELEASE
EMBARGOED UNTIL SUNDAY 8 JULY, 17.15 CEST/16.15 BST

GENOME-EDITING TOOLBOX TO TREAT HUNTINGTON'S DISEASE

Genome-editing is showing promise for treating Huntington's disease, say Swiss neuroscientists. They have found a way to disrupt the damaging effects of a faulty gene that causes this devastating disease.

Huntington's disease is an inherited condition that affects around one in 10,000 people worldwide. The faulty (mutated) gene attacks the nervous system leading to abnormal movements and changes in cognition and emotions. It is a very challenging disease to live with, and there is no cure, although some drugs can help relieve the symptoms.

Professor Nicole Déglon from Lausanne University Hospital told delegates today (8 July) at the FENS Forum of European Neuroscience in Berlin, "Our research demonstrates that genome-editing tools provide a valuable approach for the treatment of Huntington's disease."

The mutation is found in the huntingtin gene which produces a misfolded protein that generates the movement and psychiatric disorders. In the later stages of the disease, large areas of the brain degenerate.

Professor Déglon and her team used the recently discovered Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) technique in mice. CRISPR allows scientists to manipulate the DNA in the cell's nucleus, acting like a pair of scissors (known as Cas) to cut – or edit - the DNA at a very precise location. They used a powerful virus system to deliver the CRISPR genes into the brains of a mouse model of Huntington's disease. Fluorescent proteins were attached to monitor the genes.

"By applying CRISPR/Cas techniques, we found that both in the cultures of the nerve cells and in the mouse brain the huntingtin gene was disrupted by as much as 90%. The misfolding of the protein was reduced which significantly slowed down the disease process," she said.

Once the CRISPR/Cas gene is in place in the brain, it stays there forever and continues to cut the DNA. The risk is that it could eventually cut other bits of DNA not linked to Huntington's disease and this could cause other problems. To overcome this, Professor Déglon has developed a form of the scissor-like Cas, known as kamiCas9 that shuts itself down when its work is done.

"These results are an important step towards the use of the CRISPR system to disrupt the damaging effects of the faulty huntingtin gene, and show us the therapeutic potential of our approach," said Professor Déglon.

Professor Déglon's team is now evaluating more complex treatment and repair strategies uing rodent models of Huntington's disease and neurons derived from human patients. "We need to identify the most efficient and safe approach to block the effects of

the mutant protein in the brain and pave the way for preclinical developments of Huntington's disease gene editing," she concluded.

END

Symposium S15: Crispr/cas9-based gene editing for modeling and treating neurological disorders
Abstracts: N. Deglon - Genome editing for neurodegenerative disorders with the self-inactivating Kamicas9 system

Contact
FENS Press Office and all media enquiries:
Elaine Snell, Snell Communications Ltd, London UK *(English language)*
tel: +44 (0)207 738 0424 or mobile +44 (0)7973 953794
email: Elaine@snell-communications.net

Barbara Ritzert, ProScience Communications, Pöcking, Germany *(German language)*
tel: +49 8157 9397-0 or mobile +49 151 12043311
email: ritzert@proscience-com.de

NOTES TO EDITORS
Nicole Déglon, Department of Clinical Neurosciences, Lausanne University Hospital, Switzerland http://www.unil.ch/lcmn

The 11th FENS Forum of Neuroscience, the largest basic neuroscience meeting in Europe, organised by FENS and hosted by the German Neuroscience Society will attract more than 7,000 international delegates. The Federation of European Neuroscience Societies (FENS) was founded in 1998. With 43 neuroscience member societies across 33 European countries, FENS as an organisation represents 24,000 European neuroscientists with a mission to advance European neuroscience education and research. https://forum2018.fens.org/