Technology Offer

pactt technology transfer

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GENE-EDITING FOR THE TREATMENT OF HUNTINGTON'S DISEASE

Huntington's disease (HD) is an orphan disease caused by brain-destroying mutant proteins which ends in dementia and death. Genetic mutation is due to a repetition of DNA triplet and affects around 1/10'000 people worldwide. Currently, no curative treatment is available.

DESCRIPTION

The invention provides a kit for the treatment of Huntington's disease using CRISPR system for blocking the expression of the mutant huntingtin (*mHTT*) or repairing the CAG expansion causing the disease. The kit (or therapeutic cassette) comprises a gene delivery vector, a human codon-optimized Cas9, one artificial single guide RNA (sgRNA) recognizing the *HTT* gene, and a system ensuring transient editing activity.

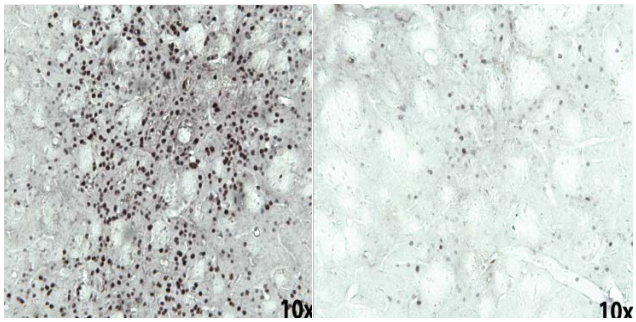
INTELLECTUAL PROPERTY

Patent application: PCT/EP2015/067986
Priority date: August 04, 2014
Applicant: Lausanne University hospital
Inventors: N. Déglon and N. Merienne

ADVANTAGES

- Novel therapeutic strategy for an orphan disease with a high unmet medical need
- Single treatment of mutant code for a permanent benefice
- High safety due to DNA targeting and self inactivation
- Use of one kit/delivery system including all components
- Gene delivery vector targeting neuronal and glial cells

TYPICAL RESULTS



Left: brain of untreated mouse, showing huntingtin protein aggregation. Right, the brain of a mouse treated with CRISPR-Cas9 editing, showing the strong reduction of misfolded huntingtin, after 3 weeks of treatment.

PROOF OF CONCEPT

In mouse primary affected HD neurons/astrocytes, and in human neurons derived fom iPS cells up to fifty percent gene disruption was achieved.

In adult mice expressing a human *mHTT* gene, the group treated with the "therapeutic cassette" containing the gene-editing enzyme Cas9 and the RNA targeting the *HTT* gene showed an extremely efficient gene disruption and up to 90% reduction of *mHTT* aggregation compared to control group (without CRISPR treatment).

STAGE OF DEVELOPMENT

Pre-clinical stage: *in vitro* and *in vivo* using cells and mouse models of Huntington's disease.

COLLABORATION OFFER

PACTT is looking for industrial partners for further development and offers to grant exclusive or non exclusive patent licenses.

REFERENCE

IDF 17/14

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