Licensing Opportunity

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Treating Huntington's disease and other trinucleotide repeat disorders with CRISPR-Cas9 nickase

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Currently, 14 neurologic disorders are known to be caused by CAG repeat expansions. These orphan diseases, including Huntington's disease, are a group of largely untreatable, heritable neurological disorders affecting 1/1'000 people worldwide. Treatments have been focused on mitigating the symptoms and does not slow the progression of these diseases. But because the size of the CAG repeat expansion determines the severity of the disease, it may be possible to contract the repeat tract and reverse the disease phenotypes.

DESCRIPTION

The inventors found a new way of inducing CAG repeat contractions using the CRISPR-Cas9 system. The kit comprises an optimized Cas 9 and a single guide RNA recognizing a target sequence. It can be delivered to any types of cells to reduce the size of the repeat tract. These are advantages over other methods currently in development that are specific to a single disease and use multiple guide RNAs, which increases the potential of off target mutations.

STAGE OF DEVELOPMENT

Preclinical studies on patient-derived cell lines have been performed.

In vivo studies in Huntington disease mice model have shown positive results

ADVANTAGES

- Orphan disease with a high unmet need
- Targets all expanded CAG repeat disorders
- Innovative and targeted therapeutic strategy
- Off target mutations below detection limit
- Contracts only the expanded allele, leaving the essential allele intact.

INTELLECTUAL PROPERTY

Patent application PCT/EP2017/058940 extended in national phases in Europe and US
Priority date: April 14, 2016
Applicant: University of Lausanne
Inventors: V. Dion, C. Cinezi, L. Aeschbach,

COLLABORATION OFFER

PACTT offers to grant exclusive or non exclusive license to industrial partners able to develop and commercialize the technology.

REFERENCE

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