Licensing Opportunity

pactt technology transfer

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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

In vitro assays on human cell lines have been done to prove the effectiveness of the method.

Preclinical studies on patient-derived cell lines and optimization of delivery system for studies in mice ongoing.

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

European Priority application in the name of University of Lausanne naming as inventors: V. Dion, C. Cinezi, L. Aeschbach,
priority date on April 14, 2016

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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