Technology Offer

ALTERNATIVE AND EFFICIENT DELIVERY METHOD OF SILENCING SEQUENCES FOR THE TREATMENT OF BRAIN DISEASES

KEYWORDS

Gene Therapy; Extracellular Vesicles; miRNA; Spinocerebellar Ataxia Type 3; Ataxin-3; Intranasal; ExoMotif; MJD; SCA3

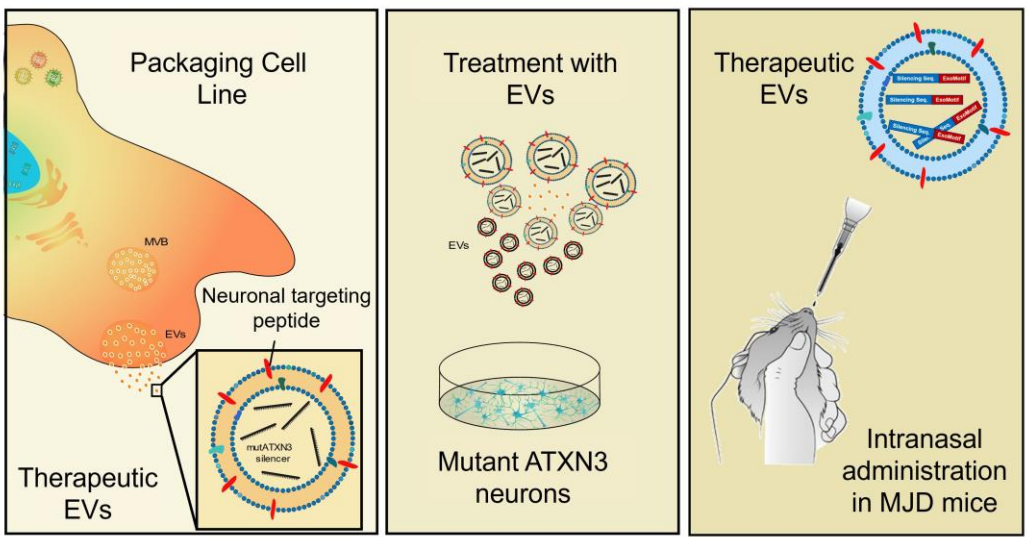
TECHNOLOGY DESCRIPTION

The **first** extracellular vesicles (EVs) as therapeutic vehicles targeting the brain containing engineered miRNA-based silencing sequences to treat Machado-Joseph Disease/Spinocerebellar Ataxia Type 3 (MJD/SCA3) upon daily intranasal administration.

Advantages:

- Packaging cell line to continuously produce Engineered EVs
- 5x more delivery due to a specific tropism for neurons
- 2.5-fold enrichment of silencing sequences into EVs due to the presence of ExoMotifs
- Non-invasive administration: Intranasal route

An alternative and efficient delivery method for miRNAs/Silencing sequences to treat MJD and other brain diseases



ADVANTAGES OVER ALTERNATIVE TECHNOLOGIES

- Packaging Cell line to large scale and continuous production of engineered EVs.
- Engineered EVs or similar vesicles for delivery of silencing sequences:

No other engineered EVs were used to treat brain disorders until now, namely MJD/SCA3

Other methods for delivery of silencing sequences:

- Intranasal (IN) vs intracranial delivery: IN delivery allows multiple administrations
- Intranasal (IN) vs intravenous delivery: IN delivery is a non-invasive route.

APPLICATIONS

Treatment of Machado-Joseph Disease
Treatment of other brain disorders

PATENT SPECIFICATIONS

Reference: PCT/IB2023/060856
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