Nonhuman adenovirus vectors for gene transfer to the brain (BRAINCAV)

https://neurodegenerationresearch.eu/survey/nonhuman-adenovirus-vectors-for-gene-transfer-to-the-brain-braincav/

Title of project or programme

Nonhuman adenovirus vectors for gene transfer to the brain (BRAINCAV)

Principal Investigators of project/programme grant

Title	Forname	Surname	Institution	Country
Dr	Eric	Kremer	CENTRE NATIONAL DE LA RECHERCHE SCIENTIFIQUE	France

Address of institution of lead PI

Institution	CENTRE NATIONAL DE LA RECHERCHE SCIENTIFIQUE
Street Address	
City	
Postcode	

Country

France

Source of funding information

European Commission

Total sum awarded (Euro)

2984999

Start date of award

01-10-2008

Total duration of award in months

48

The project/programme is most relevant to

- Parkinson’s disease

Keywords

Research abstract in English

Formidable challenges remain to prevent and treat successfully neurodegenerative diseases. Traditional pharmacological approaches, as well as those using stem cells, have made progress but

their impact remain limited. As suggested by clinical results in Canavan and Parkinson’s disease, gene transfer offers substantial potential. However, this strategy of therapeutic intervention also brings unique obstacles – in particular the need to address feasibility, efficacy and safety. BrainCAV’s foundation is the potential of canine adenovirus type 2 (CAV-2) vectors, which preferentially transduce neurons and undergo a very efficient long-distance targeting via axonal transport. Moreover, the episomal long-term expression leads to safe, efficient neuron-specific gene delivery. We proposed a structured translational approach that spans basic research through pre-clinical model feasibility, efficacy and safety. To provide a proof-of-principle of the effectiveness of CAV-2, we tackle mucopolysaccharidosis type VII, a global, orphan disease commonly affecting children, and Parkinson’s disease, a focal degeneration of dopaminergic neurones commonly affecting aged population. To develop and execute this project, BrainCAV brings together an interdisciplinary combination of partners with unique expertise that will take CAV-2 vectors to the doorstep of clinical trials.

Lay summary