

Advanced gene therapy tools for treatment of CNS-specific disorders (NEUGENE)

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Title of project or programme

Advanced gene therapy tools for treatment of CNS-specific disorders (NEUGENE)

Principal Investigators of project/programme grant

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Source of funding information

European Commission

Total sum awarded (Euro)

3000000

Start date of award

01-10-2008

Total duration of award in months

36

The project/programme is most relevant to

- Parkinson's disease
- Neurodegenerative disease in general

Keywords

Research abstract in English

Curative therapies still do not exist for most CNS diseases but gene therapy is a promising new approach. We propose that it will be possible to modify brain function and pathophysiology by targeted delivery of specific curative factors to selected populations of brain cells that are affected by disease. This opens the door for effective treatment regimes, which can be tailored to individual patients needs.

However, currently available gene transfer vectors have limitations regarding safety and efficacy, as they do not allow for targeting of specific populations of neurons or glia or regulation of transgene expression. The NEUGENE consortium has been founded by leading European scientists from academia and industry to overcome these limitations. The consortium will develop Adeno-associated virus (AAV) and Lentivirus (LV)- based tools for targeted and regulated gene transfer into different populations of CNS cells. The consortium will provide a selection of vectors that are optimized for different therapeutic approaches, e.g. regulated expression of neurotrophic factors or manipulation of neurotransmitter synthesis in specific neurons.

Lay summary