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ProGen:Protecting against neurodegeneration by somatic gene therapy

Project Description

Alzheimer´s disease (AD) is an incurable neurodegenerative dementing disorders of higher age with an enormous socio-economic burden. The lifetime risk for AD between 65 and 100 years is 33% for men and 45% for women. By 2025 about one-third of the European population will be older than 65 years and, thus, be at risk for dementing disorders. Currently, there is neither an effective prevention nor a treatment available. AD is one of the leading causes of disability, and represents the fastest growing area of unmet medical need. The number of demented patients in Europe, currently about 8 million will double to triplicate within the next 20 years. Medicare costs will rise accordingly. Annual costs of over € 130 billion in the EU already today makes AD the third most expensive disease. An intervention that could delay the onset of AD by five years would already cut the number of patients with AD by half.

Here we propose to establish and validate under experimental conditions in animals a gene therapeutic approach that will slow down or even prevent neurodegeneration with high therapeutic efficacy and minimal or no side-effects. The therapeutic approach is based on a new concept that targets critical molecular switches that under neurodegenerative conditions regulate aberrantly activated cell division mechanisms in neurons. Therapeutic tools to prevent neurodegeneration will subsequently be transferred to clinical applications. Principal solutions to be developed in this project can be applied with only minimal modifications to a variety of other neurodegenerative CNS disorders including Parkinson´s disease or Amyothrophic Lateral Sclerosis.

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