Developing a SCA1 therapy targeting the ataxin-1 protein in a patient-specific neuronal cell model

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Country

Netherlands

Title of project or programme

Developing a SCA1 therapy targeting the ataxin-1 protein in a patient-specific neuronal cell model

Source of funding information

Hersenstichting

Total sum awarded (Euro)

€ 160,000

Start date of award

01/09/2016

Total duration of award in years

4

Keywords
Research Abstract

The goal of this project is to develop three different strategies to reduce toxicity of the polyglutamine expanded ataxin-1 protein that causes spinocerebellar ataxia type 1(SCA1), a neurological condition that leads to progressive disturbances of coordination, balance, speech and swallowing, and to loss of independence and early death. SCA1 is an autosomal dominant disorder caused by an expansion of a CAG repeat in the ataxin-1 gene and mRNA that results

in an expanded polylgutamine repeat in the ataxin-1 protein. This protein with an expanded polyglutamine stretch aggregates in the brain, and these protein aggregates are considered a pathological hallmark of the disease.

We hypothesize that by prolonged lowering of mutant ataxin-1 toxicity, we can halt or reverse the disease progress in SCA1 patients. We will reduce toxicity by designing different antisense oligonucleotides (AONs) that specifically target the disease-causing ataxin-1 mRNA, resulting in either reduced expression of the ataxin-1 protein, or in a modified ataxin-1 protein lacking the polyglutamine expansion. At the end of this project, we will select the two most promising therapeutic strategies for further development.

Further information available at:

Types:
Investments < €500k

Member States:
Netherlands

Diseases:
N/A

Years:
2016

Database Categories:
N/A

Database Tags:
N/A