

# RNA editing for treatment of Huntingtons disease

<https://neurodegenerationresearch.eu/survey/rna-editing-for-treatment-of-huntingtons-disease/>

## Principal Investigators

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Lund University

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### Country

Sweden

## Title of project or programme

RNA editing for treatment of Huntingtons disease

## Source of funding information

Swedish Research Council

## Total sum awarded (Euro)

€ 1,088,139

## Start date of award

01-01-2012

## Total duration of award in years

5

## The project/programme is most relevant to:

Huntington's disease

## Keywords

### Research Abstract

Huntington's disease (HD) is a hereditary neurodegenerative disorder caused by an expanded CAG repeat in the huntingtin (htt) gene. Individuals with the mutant htt gene suffer throughout their adult life and die in agony at midlife. Available medication provides only minor symptomatic relief. There is therefore an urgent need for the development of effective treatments. Our aim is to develop a completely novel and groundbreaking therapy based on an mRNA editing principle

called trans-splicing, which will correct the mRNA coding for the mutated htt protein to make wild type htt instead. Our chosen strategy to correct mutant htt into wild-type htt compared to only reducing mutant htt may be critical as normal htt is essential for neuronal function. To develop and evaluate this therapy, we will use unique cell models based on human cells and animal models expressing the entire human genomic region for the htt gene. The therapeutic tool is an adeno-associated virus vector, which shows excellent safety for gene delivery in the brain. We will also employ novel detection tools for quantification of mutant and corrected htt proteins and advanced magnetic resonance imaging and spectroscopy techniques in both animals and HD patients using the state-of-the-art equipment at Lund University Bioimaging Center. Relevant ethical and social aspects with respect to novel treatment strategies will be incorporated and taken into consideration at an early stage in the project.

### **Lay Summary**

**Further information available at:**

#### **Types:**

Investments > €500k

#### **Member States:**

Sweden

#### **Diseases:**

Huntington's disease

#### **Years:**

2016

#### **Database Categories:**

N/A

#### **Database Tags:**

N/A