DEVELOPING A MICRORNA-TARGETED THERAPY FOR ALS

https://www.neurodegenerationresearch.eu/survey/developing-a-microrna-targeted-therapy-for-als/

Principal Investigators

MILLER, TIMOTHY M

Institution

WASHINGTON UNIVERSITY

Contact information of lead PI
Country

USA

Title of project or programme

DEVELOPING A MICRORNA-TARGETED THERAPY FOR ALS

Source of funding information

NIH (NINDS)

Total sum awarded (Euro)

305045.8716

Start date of award

15/04/2012

Total duration of award in years

1

Keywords

Amyotrophic Lateral Sclerosis, Antisense Oligonucleotides, targeted treatment, MicroRNAs, Spinal Cord

Research Abstract

DESCRIPTION (provided by applicant): Amyotrophic lateral sclerosis is characterized by the progressive loss of motor neurons in the spinal cord, resulting in stiffness, severe weakness, atrophy of skeletal muscles, and eventual death from respiratory failure in 3-5 years. There are no current therapies that substantially slow the progression of the disease. In animal models and in samples from ALS patients, we have discovered changes in small non-coding RNA

called microRNAs. We will now validate one particular microRNA as a therapeutic target and develop a method of inhibiting this microRNA using antisense oligonucleotides. We hypothesize that inhibition of this miRNA will substantially slow ALS in animal models. Given our current experience in Phase I trial using antisense oligonucleotides in ALS patients; we intend to translate our findings from this grant to a novel therapeutic for ALS.

Further information available at:

Types:
Investments < €500k

Member States:
United States of America

Diseases:
N/A

Years:
2016

Database Categories:
N/A

Database Tags:
N/A