Development of a small molecule therapeutic for the treatment of prion infection of humans

https://www.neurodegenerationresearch.eu/survey/development-of-a-small-molecule-therapeutic-for-the-treatment-of-prion-infection-of-humans/

Principal Investigators

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Institution

MRC Prion Unit

Contact information of lead PI
Country

United Kingdom

Title of project or programme

Development of a small molecule therapeutic for the treatment of prion infection of humans

Source of funding information

MRC

Total sum awarded (Euro)

€ 686,993

Start date of award

26/08/2014

Total duration of award in years

2.5

The project/programme is most relevant to:

Prion disease

Keywords
Research Abstract

Prion diseases are rapidly progressive, invariably fatal, neurodegenerative conditions for which there is currently no treatment. Although rare, they are seen as prototypic neurodegenerative diseases of protein misfolding and also as tractable for therapeutics. Laboratory animals are naturally susceptible to prion diseases and so therapeutics can be tested against the disease itself rather than in animal models of uncertain validity. As part of a unique collaboration with

GlaxoSmithKline we have identified and progressed a series of compounds that dramatically lower prion levels in cell assays. Significantly, tool compounds from this lead series are effective when administered orally in the treatment of prioninfected mice with well-established neuropathology. In sharp distinction to other neurodegenerative diseases, where therapeutic biomarkers are of uncertain relevance to underlying disease mechanisms or activity, we are able to follow the infectious prion titre directly in mice using a resource unique to the MRC Prion Unit, an automated cell-based prion bioassay, such that such studies are more akin to those of HIV trials for example, where effect of drugs on viral load can be studied. Neurodegenerative dementias pose a massive and increasing challenge to healthcare systems and these studies provide an unique opportunity to target a rare but clearly defined human dementia: it is anticipated much may be learned of wider relevance for the commoner neurodegenerative diseases, all of which involve accumulation of misfolded host proteins. We now propose to complete optimisation of this lead series to deliver a clinical candidate compound.

Lay Summary
Further information available at:

Types:
Investments > €500k

Member States:
United Kingdom

Diseases:
Prion disease

Years:
2016

Database Categories:
N/A

Database Tags:
N/A