

# NovaUCD

## **Technology Transfer Opportunity**

### **Treatment of Prion Diseases**

#### **OPPORTUNITY:**

Treatment of prion diseases.

#### **Description of Technology:**

The present invention relates to a compound that potentially may be used in the treatment of prion diseases.

#### Value Proposition:

Prion diseases, also known as transmissible spongiform encephalopathies (TSEs), are a group of fatal neurodegenerative disorders that affect humans and animals. Human prion diseases include kuru, Gerstmann-Straussler Scheinker syndrome (GSS), Fatal Familial Insomnia (FFI) and Creutzfeldt-Jakob Disease (CJD). Scrapie in sheep and goats and Bovine Spongiform Encephalopathy (BSE) in cattle are among the animal prion diseases.

Infected hosts incubate TSE for months to decades and their health declines rapidly after the onset of clinical symptoms. Death invariably follows within a period of months.

Prion diseases are so-called because the prion protein is considered to be central to the development of the disease. The prion protein exists in at least two different conformational forms - a normal cellular form of the prion protein known PrPC and is an abnormal pathological form known as PrPSc. It is thought that conversion from PrPC to PrPSc is a critical event in prion disease formation and development as PrPSc tends to aggregate in the brain thus resulting in the characteristic neuropathological features.

Currently, there is no known cure for prion diseases. Standard approaches to treat the disease have proved ineffective or even dangerous, due to an inability of the compounds under investigation to cross the blood brain barrier and/or due to the toxic side effects. The compound discovered by UCD researchers acts by preventing or reducing the conversion of PrPC to PrPSc thus potentially preventing or ameliorating prion disease. Importantly, this compound has been shown to cross the blood brain barrier and has been approved for use in other medical applications.

#### Market:

Pharmaceutical and veterinary market sectors.

#### **Inventors**:

Dr Hilary McMahon and Marguerite Prior, UCD School of Biomolecular and Biomedical Science.

#### **Publications:**

Prior M, et al. (2007): "Cyclodextrins inhibit replication of scrapie prion protein in cell culture", Journal of Virology, Oct. 2007, p. 11195-11207.

#### Status:

The invention is based on pre-clinical in vitro data. A priority patent application was filed in May 2006 and this application entered PCT in May 2007, International Publication Number WO2007/137808.

#### **Opportunity Sought:**

The invention is available for licensing and/or collaborative research.

#### **Contact:**

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