Monash University researchers have identified a microRNA as a possible treatment for neurodegenerative diseases associated with protein aggregation (i.e. Huntington’s, Parkinson’s disease).

- Conserved microRNA between species
- Demonstrated prevention of protein aggregation in Huntington’s and Parkinson’s disease models.
- Regulates proteostasis and autophagy pathways

**THE CHALLENGE**

Several degenerative human pathologies, including Huntington’s and Parkinson’s diseases manifest with the appearance of toxic insoluble protein aggregates. Protein aggregation is a failure of the proteostasis machinery, causing misfolding and polymerization of the protein into aggregates and organized fibrils.

Huntington’s disease is a polyglutamine-associated neurodegenerative disease caused by a mutation in the HTT gene coding for the Huntingtin protein (HTT). It has a global prevalence of 2.71 per 100,000 of the population.

There is still no cure despite many attempts to develop treatments. Among these, efforts in treating Huntington’s disease focus on:

(i) use of Rapamycin as a method of clearing protein aggregates by upregulating proteasome activity and,

(ii) use of RNA interference including synthetic siRNAs or antisense oligonucleotides to silence the mutant Huntingtin gene.

However, many questions remain regarding these approaches, notably the mechanisms that control the proteasome activity, level of safety (including off-target effects), toxicity, potential unintended consequences of lowering gene expression, and any long-term effects.

There is therefore a clear need to develop new approaches for the treatment of diseases associated with protein aggregation such as Huntington’s, Parkinson’s and Alzheimer’s diseases.

**THE TECHNOLOGY**

The Monash team led by A/Prof Roger Pocock has identified and validated in animal models (C. elegans) and mammalian cells, a conserved microRNA that prevents toxic protein aggregation.

Specifically, the team demonstrated the aggregation of both alpha-synuclein and polyglutamine (hallmark of Parkinson’s and Huntington’s disease) is regulated by the microRNA.

They also show the same microRNA prevents aggregation of huntingtin in mammalian cells.

Moreover, they demonstrated that the microRNA is required to control proteostasis and autophagy; two major disease-relevant pathways confirming the functional role of the microRNA in clearing toxic protein aggregates.

**Intellectual property:** Provisional application on microRNA treatment is pending.

**THE OPPORTUNITY**

Monash University seeks a commercial partner with expertise in therapeutic development for neuronal diseases.

The Monash team has extensive expertise in microRNA biology, neuroplasticity, stress responses and neuronal development mechanisms disease models (C. elegans) of Huntington’s, Parkinson’s and Alzheimer’s Diseases.