HUDSON INSTITUTE OF MEDICAL RESEARCH

COMMERCIALISATION OPPORTUNITY

XYnapse Therapeutics is a spin off company seeking capital investment to fund the development of novel Parkinson's Disease therapies.

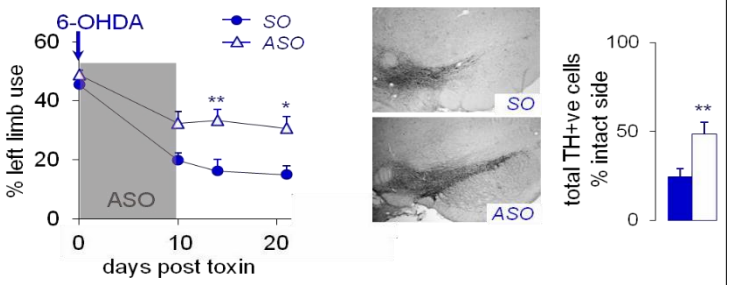
SRY inhibition to treat Parkinson's Disease in males

Background

Approximately two thirds of Parkinson's Disease (PD) patients are men. Sex-specific genes in the brain may underlie this male bias in PD. In healthy male dopamine neurons, the Y chromosome gene product, SRY (Sex-determining Region Y), modulates dopamine synthesis and motor function. Researchers at the Hudson Institute discovered that SRY expression is abnormally elevated in animal models of PD. When PD model animals were treated with *SRY antisense oligonucleotide (ASO)* treatment, SRY levels were reduced, and improved motor deficits and diminishment of death of dopamine cells were observed. These studies suggest that **lowering SRY levels by brain delivery of SRY ASOs in rat models of PD was highly therapeutic.**

Key Data

a. Motor function b.Dopamine cells



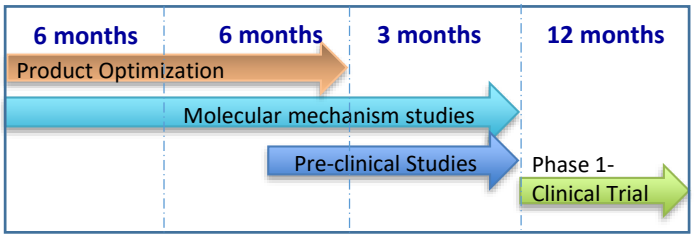
Antisense oligonucleotide treatment significantly improved motor function (A) and diminished dopamine cell loss (B) in the 6-OHDA-induced model of Parkinson's disease *6-OHDA, 6-hydroxydopamine toxin; ASO, antisense oligonucleotide (SRY treatment); SO sense oligonucleotide (control) treatment.* Patent serial number: WO2016/164977.

IP position

Patent at PCT Phase - covering SRY for all neurological diseases; including antisense oligonucleotide, filed 2015. Future IP in novel compound(s) following success of pre-clinical studies / proof-of-concept clinical trial.

Research & Development Plan

Proposed timeline for each project milestone



Current Treatment – Unmet Need

There are *no drugs on the market currently that halt the cell death associated with PD*.

Current treatments for late stage PD include Deep Brain Stimulation, in which an ASO treatment might be part of a combination therapy.

A Phase 1 study showed ASOs were injected into the cerebrospinal fluid, crossed the blood-brain barrier, and shown to be safe. *Lancet Neurol. 2013 May ; 12(5): 435–442O*

Our SRY ASO approach aims to reduce or halt the nigral cell death and therefore reduce or prevent the worsening of motor symptoms.

Global Market Opportunity

- Target market: Men with late stage PD
- Market size: 700,000 men in the USA
- $10 billion health care burden (PD association 2013)
- As the elderly population increases, this market is expected to grow further

Commercial Opportunity

XYnapse Therapeutics Pty Ltd, is an early stage biotechnology company spun-out from the Hudson Institute and is focused on development of an antisense oligonucleotide therapy targeting SRY for men with PD. The opportunity is structured to maximize non-diluting tax rebates and government incentives for R&D active Australian businesses.

Value Proposition

- Unique drug target, animal models, mechanism of action with a demonstrated effect on disease
- Antisense downregulation shown to be successful in other related neurological diseases
- World leaders in SRY research with 25 years' experience, developing unique models and human and rodent SRY antibodies
- Surgical neurology collaboration with PD treatment and trials expertise
- Access to laboratory and clinical facilities to sponsor research through to Phase 1
- Clear R&D strategy for achieving project milestones

For further information

Rob Merriel
Chief Commercialisation Officer
Hudson Institute of Medical Research
e: rob.merriel@hudson.org.au
t: +61 418 186 265
w: http://www.hudson.org.au/commercialisation