Preventing neurodegenerative disease progression

AUSTRALIA’S AGEING POPULATION

Neurodegenerative disorders are having a profound impact on the global community; on the lives of Australians and on the national healthcare system. In 2010, dementia was the third leading cause of death for Australians and a $2 billion cost to the healthcare system. With Australia’s ageing population, there is a real need to better manage these disorders.

With many neurodegenerative disorders, any available therapy is limited to short term symptom management. Unfortunately this is the case for Alzheimer’s disease, Parkinson disease and Huntington disease, yet such conditions may afflict patients for between 10 to 20 years post diagnosis.

CAN NEURODEGENERATIVE DISEASES BE STOPPED IN THEIR TRACKS?

Many neurodegenerative disorders are caused by the aggregation of certain proteins within the brain, preventing normal brain function. Enter Prana Biotechnology – an ASX and NASDAQ listed company was founded based on the work of The University of Melbourne’s Professor Colin Masters and international collaborators in discovering the role that metals played in causing the toxic aggregation of β-amyloid, the key protein involved in Alzheimer’s disease.

Prana’s metal protein attenuating compounds (MPAC’s) potentially hold the key to preventing the protein aggregation of other target proteins such as mutant huntingtin protein in Huntington disease and α-synuclein in Parkinson’s disease. Accordingly, the MPAC therapeutic strategy promises to be potentially disease modifying across several neurodegenerative disorders.

OUTSOURCING IS THE KEY TO SUCCESS FOR SMALL BIOTECH COMPANIES

Prana’s Principal Scientist, Jack Parsons, worked closely with VPTN members to support the creation, development and progression of Novel Chemical Entities (NCE’s) into Preclinical Development.

Drug development is required to characterise and establish the physicochemical properties of the NCE, including its identity, stability and purity. Pharmacokinetic profiling and ADME (Absorption, Distribution, Metabolism and Elimination) studies allows for an understanding of how the NCE can perform as a drug in a biological organism.

Accessing leading-edge technology and services, Prana researchers worked with two facilities delivering analytical chemistry services located at the Bio21 Institute [Melbourne Mass Spectrometry and Proteomics and Melbourne Magnetic Resonance facilities] and the pharmacokinetic analysis conducted by the Centre for Drug Candidate Optimisation (CDCO). Drawing on each facility’s capabilities, Prana’s potential drugs are profiled, resulting in data that inform critical decisions to progress the development cycle of new neurodegenerative medicines.
As a small biotech company, installing the latest technology was not financially viable. So tapping into a facility network to outsource key steps in the discovery pathway, allowed Prana to focus its capital expenditure on its people and equipment that is critical to any discovery pipeline.

With one compound, PBT2 in Phase 2 clinical trials for Huntington’s Disease treatment, and another exciting novel chemical entity in preclinical development, Prana’s MPACs offer new hope to patients and the chance of a better, healthier life post-diagnosis.

EVIDENCE OF A NEW PARADIGM

In establishing potential medicines that are both safe and effective is a detailed journey. Prana’s lead candidate PBT2 needs to complete Phase 3 clinical studies to be approved by regulatory bodies to treat Huntington’s or other neurodegenerative disorders. However, evidence suggests that Prana’s potential drug and related molecules could represent a new paradigm for the treatment of patients in slowing the progression of neurodegenerative diseases.

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To further support these therapies, in May 2016, Prana Biotechnology received a $6.5 million cash refund under the Australian Government’s R&D Tax Incentive scheme. These funds will be used to further Prana’s development of PBT2 for the treatment of Huntington disease and PBT434 for the treatment of various atypical Parkinsonian movement disorders.

ABOUT THE VPTN

Established in 2009, the Victorian Platform Technologies Network (VPTN) plays a key role in connecting publicly-funded facilities in Victoria with industry and researchers. Through its online services – ARIN and PlatformConnect – VPTN is unique in offering a centralised, open and cross-institutional network of over 160 platform technologies across more than 30 institutions. VPTN is realising its vision by linking innovation with technology and expertise in biological, materials, engineering, physical, chemical, food, sports, information, nano, design and mathematical sciences. The VPTN is an initiative supported by the Victorian Government, Biomedical Research Victoria and Monash University. Discover more at platformtechnologies.org.