

## **Prana to commence Phase 1 clinical trial of PBT434 for treatment of parkinsonian diseases**

### **Highlights:**

- **Prana receives ethics committee approval for a clinical trial evaluating first in human dosing of PBT434**
- **Recruitment has commenced for the first cohort of healthy volunteers**
- **Study conducted by leading Australian early phase clinical trial facility Nucleus Network, in Melbourne, Australia**
- **PBT434 has been shown to prevent  $\alpha$ -synuclein accumulation and neuron loss in experimental animal models of Parkinsonian diseases**

**MELBOURNE, AUSTRALIA AND SAN FRANCISCO, USA – June 14, 2018:** Prana Biotechnology Ltd (ASX PBT: NASDAQ PRAN) is pleased to announce it has received ethics committee approval and has commenced recruitment for its Phase I Clinical trial evaluating the safety, tolerability and pharmacokinetics of the Company's lead drug candidate, PBT434, in healthy volunteers.

PBT434 is the first of a new generation of small molecules designed to inhibit the aggregation of alpha( $\alpha$ )-synuclein and tau, vital intracellular proteins that are implicated in neurodegenerative diseases such as Parkinson's disease and atypical parkinsonism. PBT434 has been shown to reduce the abnormal accumulation of these proteins in animal models of disease by restoring normal iron balance in the brain.

Multiple System Atrophy (MSA) and Progressive Supranuclear Palsy (PSP) are two forms of atypical parkinsonism with no approved therapies and Prana's initial targets for PBT434. Sufferers experience especially rapid deterioration compared to Parkinson's disease and typically have motor symptoms that respond poorly to available treatments. Patients with MSA also have difficulty maintaining their blood pressure along with bowel and bladder dysfunction whereas PSP patients have unsteady gait, frequent falls, visual difficulties and cognitive impairment.

The trial is being conducted by the Nucleus Network in Melbourne, Australia, and will recruit healthy adult and elderly volunteers, with the primary goal of assessing the safety and tolerability of PBT434 after single and multiple oral dose administration. Secondary endpoints include a range of pharmacokinetics measures to understand how PBT434 is absorbed and metabolised in the body.

The volunteers in the single ascending dose portion of the study will receive one single oral dose of PBT434 and will be monitored for 72 hours for safety and blood levels of drug. In the repeated dose portion of the study, subjects will receive eight days dosing with PBT434 with safety and testing for drug disposition over this time.

Prana's Chief Medical Officer and Senior Vice President, Clinical Development Dr David Stamler, MD, said: "We are excited to begin this important phase of clinical evaluation of PBT434. Following successful completion of this study, we aim to evaluate PBT434 in MSA and PSP, which are devastating neurodegenerative diseases with no approved therapies."

Parkinsonian movement disorders affect around 10 million people worldwide, and are best known for the impairment of motor function, gait, balance and cognition. Both MSA and PSP are orphan diseases in the US and Europe, two of the largest potential markets for PBT434.

Nucleus Network is a highly regarded clinical trial site with a strong network of volunteers to support recruitment of the study.

Prana will update the market on the progress of the clinical trial at significant events including first patient dosing and trial completion.

### Clinical Appendix

Study title	A Phase 1 Single and Multiple Ascending Dose Study of PBT434 in Healthy Volunteers
Unique identifier	PBT434-101
Primary endpoint	To assess the safety and tolerability of PBT434 after single and multiple oral dose administration
Secondary endpoints	<ol style="list-style-type: none"> <li>1) To determine the pharmacokinetics (PK) of PBT434 and metabolites after single and multiple oral dose administration</li> <li>2) To evaluate the pharmacokinetics of PBT434 and metabolites after multiple oral dose administration in healthy elderly subjects</li> <li>3) To evaluate the preliminary effect of food on the pharmacokinetics of PBT434</li> </ol>
Study design	This is a Phase 1, single-centre study consisting of two parts, both of which are randomised, double-blind, and placebo-controlled: The single ascending dose (SAD) and the multiple ascending dose (MAD) parts of the study will be conducted in sequential stages in healthy volunteers. Up to six single dose cohorts and four repeated dose cohorts will be evaluated.
Populations	Healthy adult volunteers age 18-55 years. Healthy elderly volunteers age ≥65 years.
Trial locations	Nucleus Network, Australia

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**About Prana Biotechnology:**

Prana's lead candidate, PBT434, is the first of a new generation of small molecules designed to inhibit the aggregation of pathological proteins implicated in neurodegeneration. PBT434 has been shown to reduce abnormal accumulation of  $\alpha$ -synuclein and tau proteins in animal models of disease by restoring normal iron balance in the brain. In this way, it has excellent potential to treat various forms of atypical parkinsonism such as Multiple System Atrophy (MSA) and Progressive Supranuclear Palsy (PSP).

For further information please visit the Company's web site at [www.pranabio.com](http://www.pranabio.com).

**Forward Looking Statements:**

*This press release contains "forward-looking statements" within the meaning of section 27A of the Securities Act of 1933 and section 21E of the Securities Exchange Act of 1934. The Company has tried to identify such forward-looking statements by use of such words as "expects," "intends," "hopes," "anticipates," "believes," "could," "may," "evidences" and "estimates," and other similar expressions, but these words are not the exclusive means of identifying such statements. Such statements include, but are not limited to any statements relating to the Company's drug development program, including, but not limited to the initiation, progress and outcomes of clinical trials of the Company's drug development program, including, but not limited to, PBT2, and any other statements that are not historical facts. Such statements involve risks and uncertainties, including, but not limited to, those risks and uncertainties relating to the difficulties or delays in financing, development, testing, regulatory approval, production and marketing of the Company's drug components, including, but not limited to, PBT2, the ability of the Company to procure additional future sources of financing, unexpected adverse side effects or inadequate therapeutic efficacy of the Company's drug compounds, including, but not limited to, PBT2, that could slow or prevent products coming to market, the uncertainty of patent protection for the Company's intellectual property or trade secrets, including, but not limited to, the intellectual property relating to PBT2, and other risks detailed from time to time in the filings the Company makes with Securities and Exchange Commission including its annual reports on Form 20-F and its reports on Form 6-K. Such statements are based on management's current expectations, but actual results may differ materially due to various factors including those risks and uncertainties mentioned or referred to in this press release. Accordingly, you should not rely on those forward-looking statements as a prediction of actual future results.*