

## **Prana receives first orphan drug designation from the FDA for the treatment of Multiple System Atrophy**

**MELBOURNE, AUSTRALIA AND SAN FRANCISCO, USA – 31st January 2019:** Prana Biotechnology Ltd (ASX: PBT, NASDAQ: PRAN) (“Prana” or “the Company”) has today announced the US Food and Drug Administration (FDA) has granted Orphan Drug designation for its lead molecule, PBT434, for the treatment of Multiple System Atrophy (MSA). This is the first time the FDA has granted orphan designation to a drug for the treatment of MSA.

Orphan Drug designation by the FDA entitles Prana to seven years of market exclusivity for the use of PBT434 in the treatment of MSA and qualifies the sponsor of the drug for various development incentives of the Orphan Drug Act, including tax credits for qualified clinical testing.

Prana’s successful application was based on the proposed use of PBT434 as a treatment for MSA, including the medical and scientific rationale. The application articulated how PBT434 prevents  $\alpha$ -synuclein accumulation, preserves neurons, and improves motor function in a widely accepted animal model of MSA. Alpha-synuclein is of great interest because aggregated forms of the protein are a pathological hallmark of Parkinsonian conditions, including MSA, and it represents a recognised therapeutic target by the scientific community.

“We are pleased that the FDA has acknowledged the importance of PBT434 as a potential treatment for MSA. This recognition, in conjunction with the recent investment from Life Biosciences, positions us strongly to accelerate the development of PBT434 for this devastating condition,” said Dr David Stamler, Chief Medical Officer.

Prana has identified a clear unmet medical need with no approved treatments specifically for MSA. Prana is conducting a Phase 1 clinical trial of PBT434 and expects it to be completed this year.

**ENDS**

**Contacts:**

Media

Scott Newstead

E: [snewstead@we-buchan.com](mailto:snewstead@we-buchan.com)

Tp: +61 429 703 014

**Investor Relations**

Rebecca Wilson

E: [rwilson@we-buchan.com](mailto:rwilson@we-buchan.com)

Tp: +61 0417 382 391

**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).

**About Multiple System Atrophy:**

Affecting about 5 in 100,000 people, MSA is a fatal neurodegenerative disease caused by the deterioration of nerve cells in specific areas of the brain and spinal cord. MSA is a form of "atypical" parkinsonism, where patients present with motor symptoms similar to Parkinson's disease (i.e., parkinsonism), yet respond poorly to anti-Parkinson's therapy. MSA patients also have prominent autonomic dysfunction (orthostatic hypotension; bowel and bladder and erectile dysfunction; altered temperature regulation) which are not addressed by available therapy and significantly impair quality of life. There are currently no medications indicated for the treatment of MSA.

**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," "would," "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 ability to close the Transaction, the use of proceeds of the Transaction, 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, PBT434, 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 financial markets, the difficulties or delays in financing, development, testing, regulatory approval, production and marketing of the Company's drug components, including, but not limited to, PBT434, 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, PBT434, 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 PBT434, 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.*