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Aussie firm likely to get orphan status for its new drug

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Singapore: Australia based Prana Biotechnology has recieved positive opinion from European Medicines Agency's Committee for Orphan Medicinal Products (COMP) for the designation of its drug candidate, PBT2 for the treatment of Huntington disease, as an orphan medicinal product to the European Commission (EC).

"We welcome the positive opinion from COMP for PBT2 to receive orphan designation to treat Huntington disease in Europe," said Geoffrey Kempler, Chairman and CEO, Prana.

Orphan designation is granted by the EC to encourage the development of medicines to treat rare diseases. Rare diseases are defined as life-threatening or chronically debilitating conditions that affect no more than five in 10,000 people in Europe.

Huntington disease is a neurodegenerative genetic disorder that affects muscle coordination and leads to cognitive decline and behavioral symptoms. The Huntington's Outreach Project for Education estimates there are between 40-100 cases of Huntington disease per million people in Europe.

In September last year the US Food and Drug Administration granted PBT2 orphan drug status for the treatment of Huntington disease.

In February, 2014 Prana's PBT2 had met its primary end point of safety and tolerability, and improved measures of cognitive performance, a secondary endpoint in its REACH2HD Phase 2 clinical trial involving 109 people with Huntington disease.