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Clinical trial for first oral drug candidate specifically developed for sleeping sickness

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The Drugs for Neglected Diseases initiative (DNDi) has announced today at the 9th European Congress on Tropical Medicine and International Health (ECTMIH) in Basel, Switzerland, the successful completion of Phase I human clinical trials for SCYX-7158 (AN5568), the first oral drug candidate specifically developed from the earliest drug discovery stage to combat human African trypanosomiasis, or sleeping sickness, a deadly parasitic disease transmitted by the tsetse fly.

The Phase I study, conducted in France, assessed the safety, tolerability, pharmacokinetics and pharmacodynamics of SCYX-7158 after single oral ascending doses in 128 healthy human volunteers of sub-Saharan origin. It allowed for the therapeutic dose to be determined at 960 mg administered once as three tablets, with a favourable safety profile. As the drug has a long half-life (400 hours), the study was extended to ensure extensive safety monitoring of the healthy volunteers up to 210 days. This pharmacological finding has the advantage of translating into prolonged exposure with just one dose. These Phase I results confirm that the drug penetrates the brain, which is crucial to treat the late stage of the disease, where the parasite crosses the blood-brain barrier and kills patients if no treatment is given. Based on the results of this study, DNDi and partners plan to proceed to pivotal Phase IIb/III studies in 2016 at sites in the Democratic Republic of the Congo (DRC), where 90% of cases occur.

'We are encouraged by the results of this important milestone for SCYX-7158, which is the fruit of collaboration and hard work of many

partners', said Dr Antoine Tarral, Head of the Human African Trypanosomiasis Clinical Programme, DNDi. 'The motivation has been the drug candidate's potential of becoming the first ever, oral-only, single-dose treatment for this deadly disease.' SCYX-7158 was discovered by Anacor Pharmaceuticals, Inc. The compound was identified through DNDi's lead optimization programme and successfully progressed through pre-clinical development in 2011.

'We are particularly excited about SCYX-7158 because it is the first drug candidate to come from the early discovery efforts of our lead optimization programme', said Dr Rob Don, Discovery & Pre-clinical Director, DNDi.

Sleeping sickness cases are decreasing but the disease remains persistent in remote, hard-to-reach areas of Africa. One of the major advancements in the treatment of the disease was the introduction of nifurtimox-eflornithine combination therapy (NECT) in 2009, developed by DNDi, Médecins Sans Frontières/Doctors Without Borders (MSF), and partners. NECT replaced an old, arsenic-based medicine, and today the vast majority of all late-stage sleeping sickness patients receive this combination as first-line treatment. Yet NECT still requires skilled staff in a hospital setting to administer the injections. Patients often travel days to get to health centres. Fexinidazole, administered for ten days with food, is currently being tested in clinical trials as an oral-only treatment that could treat all stages of the disease. SCYX-7158, if successful, would have the additional benefit of its unique single-dose, simple oral tablet administration. Recruitment for patient trials is targeted to begin in 2016 at remote sites in the DRC, where DNDi has been carrying out fexinidazole clinical trials.

Provided by Drugs for Neglected Diseases Initiative