Theranexus has obtained an orphan drug designation from the FDA for THN102 in the treatment of narcolepsy

A first step towards the company's ambition of securing development and marketing of THN102 in the USA.

Lyon, October 4th 2016 – Theranexus, a clinical-stage biopharmaceutical company, announced today that it's most advanced drug candidate, THN102, has received an Orphan Drug Designation (ODD) from the American regulatory agency, the FDA, for the treatment of narcolepsy, a rare and highly debilitating sleep/wake disorder.

The Orphan Drug Designation (ODD) may be granted to drugs that are intended to improve the quality of life of patients affected by rare diseases and conditions that affect less than 200,000 people in the United States. This designation grants Theranexus some interesting incentives: especially, 7 years of exclusive marketing rights in the United States upon FDA approval, as well as an exemption from certain registration fees.

"We are very happy that this first exchange with the FDA has been fruitful. This first step attests to our desire to secure regulatory development of THN102, both in Europe and in the United States," declared Franck Mouthon, Theranexus' Chief Executive Officer.

"Obtaining orphan drug status from the FDA for THN102 reaffirms the significant need for a safer and more effective drug to improve the treatment of narcolepsy patients," added Françoise Brunner, Theranexus' Chief Medical Officer.

As a drug candidate, the positive results of THN102 during a proof-of-concept study in healthy, sleep-deprived volunteers were recently presented in partnership with the French Armed Forces Biomedical Research Institute (IRBA), during the latest congress held by the European Sleep Research Society (ESRS 2016). Theranexus has initiated a Phase II study in patients with narcolepsy with the intent of demonstrating the superior efficacy of THN102 as compared to the reference treatment. Patient recruitment for this study should begin shortly.

About narcolepsy

Narcolepsy, also known as Gelineau's disease or Gelineau's syndrome, is a rare neurological disease. It is characterised by excessive, uncontrollable daytime sleepiness that is frequently associated with cataplexy (abrupt loss of muscle tone triggered by emotions), sleep paralysis, hypnagogic hallucinations, and changes in night sleep.

The disease generally appears in adolescence, both in men and women, but may also appear later (around age 35). In France, it is estimated that between 8,000 and 10,000 people are affected. More than 500,000 people are concerned worldwide. Between 50-70% of patients treated today with a wakefulness medication still suffer from sleepiness. Thus, it is essential to find a new, more effective treatment for this problem of hypersomnia which very strongly impacts the quality of life of these patients.

About THN102

THN102 is the most advanced drug candidate in the Theranexus portfolio and is the first to represent a new class of innovative therapeutic combinations that associate a neural activity modulator, in this case modafinil (Provigil®, Alertec®, Modavigil®), with a glial connexin modulator which, in the case of THN102, is low-dose flecainide. THN102 has shown its superior efficacy compared to modafinil alone in *in vivo* narcolepsy models (Duchêne et al. 2016), as well as in healthy, sleep-deprived volunteers (ESRS, September 2016). THN102 is now being studied in a Phase II clinical study in narcolepsy patients.

About Theranexus

Founded in 2013, Theranexus is a biopharmaceutical company that originated from the French Alternative Energies and Atomic Energy Commission (CEA) - an institution that has been recently recognised as one of the most innovative research organisations in the world (according to the 'Top 25 Global Innovators' ranking by Reuters in March 2016). Theranexus designs and develops innovative combinations of CNS drugs with marketed medications that have been repositioned as glial connexin modulators. The clinical objective is to improve the efficacy and safety of established neurological and psychiatric treatments.

The scientific concept developed by Theranexus offers a unique opportunity to accelerate clinical development by using well-known, registered products and to limit the risk of treatment failure for high-potential neurological and psychiatric indications for which the authorised and established treatments present difficulties linked to tolerance, efficacy, or lifecycle management in terms of patent expiry. Theranexus is supported by investment institutions (Auriga Partners, Sofimac Partners, CEA Investissement, and Rhône-Alpes Création).

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