



Theranexus

SHIFTING THE LINES AGAINST
CENTRAL NERVOUS SYSTEM
DISORDERS



Theranexus extends the capabilities of Neurolead, its active agent screening platform, to autophagy and neurological lysosomal storage disorders

Lyon, 9 March 2021 – Theranexus, a biopharmaceutical company innovating in the treatment of neurological diseases and pioneer in the development of drug candidates modulating the interaction between neurons and glial cells, today announced that it has extended the capabilities of Neurolead, its drug candidate discovery platform, to include autophagy and neurological manifestations of lysosomal storage disorders.

Neurolead, which combines the latest innovations in neuroscience and artificial intelligence, is a platform for the generation of new drug candidates based on a unique concept, the therapeutic targeting of interactions between glial and neuronal cells. By applying Neurolead to autophagy and neurological forms of lysosomal storage disorders, Theranexus is taking its research in the direction of exploring a therapeutic avenue of major interest with clear medical and economic potential.

The common characteristic of lysosomal storage disorders is a genetic deficiency that leads to a dysfunction of lysosomes, cellular organelles responsible for the degradation of several biomolecules. With its drug candidate BBDF-101 for Batten disease, Theranexus is already working on a juvenile lysosomal storage disorder characterized by an accumulation of lipoproteins in the brain.

The Neurolead platform has already led to the development and validation of various cellular models for research into autophagy, the biological process by which lysosomes recycle biomolecules. Using screening methods, Theranexus has identified diverse drug structures with the ability to impact cellular autophagy and lysosome function by various means. This research is also backed up by complementary *in silico* approaches, combining artificial intelligence with searches in vast databases to improve the characterization of the classes of structures identified.

"The results of this research, based on our models of interactions between neurons and astrocytes in a pathological context, open up new avenues for the discovery of drugs modulating autophagy. This work will enable us to reinforce the industrial potential of our platform to discover solutions suited to the strong needs of patients suffering from lysosomal storage disorders." **explains Mathieu Charvériat, Theranexus Chief Scientific Officer.**

"In France, 50 varieties of lysosomal storage disorders affect more than 3,000 adults and children. Each year, 150 new cases are diagnosed. These disorders result in the emergence of lesions in various organs including the bones, heart, lungs, liver, spleen and brain, causing particularly severe, irreversible damage. There are currently just 14 state-of-the-art treatments in France for 11 lysosomal storage disorders, which is not nearly enough considering that these disorders often lead to multiple disabilities and may be life threatening. We desperately need new treatments to overcome these diseases." **adds Stéphane Antolin, President of the Overcoming Lysosomal Storage Disorders Association (VML).**

Next financial publication:**Wednesday 7 April 2021** : 2020 Annual financial results and update on cash position on 31 March 2021**ABOUT THERANEXUS**

Theranexus is a clinical-stage biopharmaceutical company that emerged from the French Alternative Energies and Atomic Energy Commission (CEA) in 2013. It develops drug candidates for the treatment of nervous system diseases.

Thanks to its knowledge of neuron and glial cell interactions, THERANEXUS is a pioneer in the design and combination of approved substances and has a solid and diversified portfolio of drug candidates in clinical-phase testing. The company's combined drug repurposing strategy based on a solid commercial footing and a capability to rapidly demonstrate its clinical worth, enables it to produce different high-value-added proprietary drug candidates, significantly reduce development time and costs, and considerably increase the chance of its drugs reaching the market.

Accordingly, THERANEXUS is well-positioned in several indications, including for Parkinson's and Batten disease, for which there is currently no treatment available.

Theranexus is listed on the Euronext Growth market in Paris (FR0013286259- ALTHX).

More information at: www.theranexus.com

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