



Theranexus and BBDF Obtain European "Orphan Medicinal Product" Designation for BBDF-101 in Batten Disease

Lyon, 21 October 2020 – Theranexus, a biopharmaceutical company innovating in the treatment of neurological diseases, and the Beyond Batten Disease Foundation (BBDF) today announced the European Commission's decision to award "orphan medicinal product" designation to the two active ingredients, trehalose and miglustat, used in the drug candidate BBDF-101 for Batten disease, a rare, fatal genetic disorder affecting the metabolism of the central nervous system for which there is currently no treatment.

Under the agreement between Theranexus and BBDF, Theranexus has an exclusive, global license for the development and commercial use of its drug candidate BBDF-101 for juvenile Batten disease. BBDF-101 is a proprietary combination of trehalose and miglustat, two active ingredients which each have a specific role and work in synergy to restore normal metabolic activity in brain cells.

"After obtaining Orphan Drug Designation from the FDA in August, we are delighted to have secured the same status for Europe. During the summer we launched preclinical studies to confirm the safety of BBDF-101 over a long exposure time, with the aim of launching the clinical program in 2021," explains **Franck Mouthon, Chairman and CEO of Theranexus**.

"Orphan" drugs are aimed at diagnosing, preventing or treating very serious or life-threatening rare diseases that affect no more than 5 in 10,000 people in the European Union. This status gives Theranexus ten years of market exclusivity in the European Union once the drug has been granted marketing authorization. The Company will also receive scientific advice from the European Medicines Agency (EMA) to ensure that it complies with European regulatory requirements and will have direct access to the centralized marketing authorization procedure. Orphan designation also exempts the Company from paying the fees associated with the submission of the marketing authorization application.

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. Theranexus identified the key role played by non-neuronal cells (also known as "glial cells") in the body's response to psychotropic drugs (which target the neurons). The company is a pioneer in the design and development of drug candidates affecting the interaction between neurons and glial cells. The unique, patented technology used by Theranexus is designed to improve the efficacy of psychotropic drugs already approved and on the market, by combining them with a glial cell modulator. This strategy of combining its innovations with registered drugs means Theranexus can significantly reduce development time and costs and considerably increase the chance of its drugs reaching the market.

The proprietary, adaptable Theranexus platform can generate different proprietary drug candidates offering high added-value for multiple indications.

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



More information at: www.theranexus.com

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