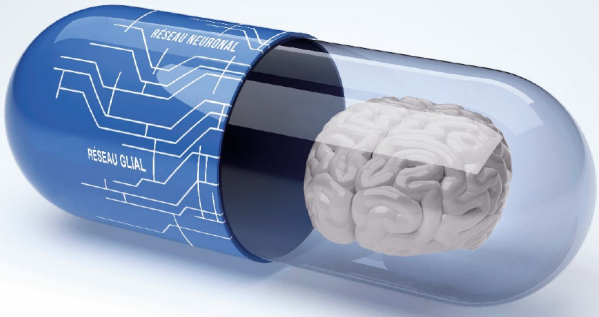
PRESS RELEASE

Theranexus

SHIFTING THE LINES AGAINST CENTRAL NERVOUS SYSTEM DISORDERS

BEYOND BATTEN DISEASE FOUNDATION



THERANEXUS AND BBDF OBTAIN ORPHAN DRUG DESIGNATION (ODD) AND RARE PEDIATRIC DISEASE DESIGNATION (RPDD) FROM THE FOOD AND DRUG ADMINISTRATION (FDA) FOR BBDF-101 FOR BATTEN DISEASE

- *Orphan Drug Designation (ODD) is a status that provides seven years of additional post-approval protection and exemption from filing fees*
- *Rare Pediatric Disease Designation (RPDD) qualifies the sponsor at the time of registration for a salable, transferable priority review voucher which can be used to speed up the approval process for another drug candidate*

Lyon, 11 August 2020 – Theranexus, a biopharmaceutical company innovating in the treatment of neurological diseases, and Beyond Batten Disease Foundation (BBDF) today announced the decision by the Food and Drug Administration (FDA) to award Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPDD) to the drug candidate BBDF-101 for Batten disease, a rare, fatal, genetic disorder of the nervous system for which there is no treatment.

In late 2019, Theranexus and BBDF signed an agreement granting Theranexus an exclusive, global license agreement for the development and commercial use of drug candidate BBDF-101 for the treatment of juvenile Batten disease. Batten disease belongs to a group of disorders referred to as neuronal ceroid lipofuscinoses (NCLs). BBDF funded research aimed at identifying and validating BBDF-101, a proprietary combination of drugs based on the synergistic effect of two active ingredients, like the other Theranexus drug candidates already in clinical development.

"We are delighted to have been awarded Orphan Drug Designation for BBDF-101 by the FDA. This is a sign of recognition for Batten disease and raises hopes for children and teens with this orphan disorder. I would like to say a big thank you to the entire BBDF team involved in the FDA submission, as well as to our donors, volunteers and the partner families of the foundation, without whom none of this would have been possible," explain **Craig Benson, Chair of the BBDF Board of Directors.**

"We are delighted to have obtained Orphan Disease Designation (ODD) and Rare Pediatric Disease Designation (RPDD). This marks a new milestone for Theranexus and BBDF in the development of the drug candidate BBDF-101. These new designations will speed up the approval process and provide at least seven years of post-approval protection and exemption from filing fees, as well as qualifying Theranexus upon approval of BBDF-101 for an assignable and transferable priority review voucher upon the registration of BBDF-101 that can be used to speed up the approval process for any other drug," continues **Franck Mouthon, Theranexus Chairman and CEO.**

The legal status of **orphan designation** in the United States was provided by the Orphan Drug Act of 4 July 1983. It qualifies the Company for accelerated review of its drug candidate for approval upon the registration - in an indication with a prevalence in the United States of less than 200 000 cases -, support with the FDA regulatory process and at least seven years of post-approval protection, as well as exemption from filing fees that normally have to be paid to the FDA.

The FDA defines **rare pediatric diseases** as rare diseases (with fewer than 200,000 cases in the United States) that are serious or life threatening and primarily affect individuals aged under 18. The aim of the program is to facilitate the development of new drugs and biological products for the prevention and treatment of rare pediatric diseases. When the FDA awards Rare Pediatric Disease Designation, the sponsor of the trial firstly is granted a 6-months accelerated review of the drug candidate, and secondly qualifies for a priority review voucher for another drug candidate irrespective of indication, and which may be sold to another company. In such case the length of the approval procedure is also considerably shortened, generally taking just 6 months instead of the usual 12 months, which gives a very high value to this priority review voucher.

After discussions with the FDA, Theranexus is currently preparing to launch a BBDF-101 preclinical trial to confirm the preclinical safety of BBDF-101 over a long exposure time, with the aim of supplementing the data already available so that the clinical program can be launched in 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. 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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