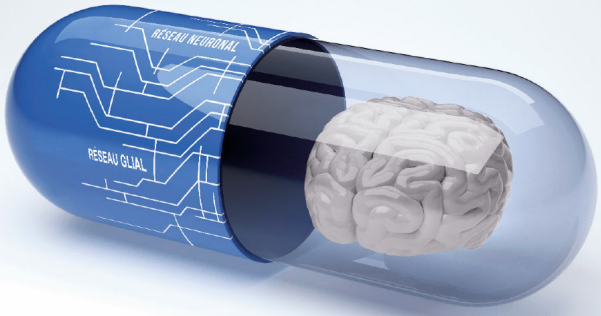
Theranexus

SHIFTING THE LINES AGAINST CENTRAL NERVOUS SYSTEM DISORDERS




# Theranexus and Beyond Batten Disease Foundation (BBDF) announce agreement in principle to develop and market Batten disease drug candidate BBDF 101

- **BBDF has funded research to identify and validate BBDF 101 for the treatment of juvenile Batten disease.**
- **Theranexus and BBDF will jointly lead the clinical development program in the US to secure regulatory approval for the product**
- **Theranexus will have an exclusive worldwide license for commercial use of the product**

**Lyon, 27 June 2019** – 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, is pleased to announce the signing of an agreement in principle (term sheet) with Beyond Batten Disease Foundation (BBDF) to lead the clinical development program aimed at securing approval and commercialization for drug candidate BBDF 101 for Batten disease. The agreement gives Theranexus a six-month exclusivity period to reach a final agreement.

Juvenile Batten disease, also known as Spielmeyer-Vogt or CLN3 disease, is a rare, fatal, inherited disorder of the nervous system for which there is no treatment or cure. Juvenile Batten disease belongs to a group of disorders referred to as neuronal ceroid lipofuscinoses (NCLs). Over 400 different errors in 13 genes have been attributed to various forms of NCL, which differ from one another primarily by when symptoms first appear. The first symptom in the juvenile form, progressive vision loss, appears between the ages of 4 and 6 which is followed by personality changes, behavioural problems, and slowed learning. Seizures commonly appear within 2-4 years of the onset of disease. Over time, patients continue to decline mentally and physically. Eventually, those affected become wheelchair-bound, are bedridden, and die prematurely. Psychiatric symptoms or psychosis can appear at any time. Juvenile Batten disease is always fatal; usually by the late teens to early 20s. In the United States and Europe, the juvenile form is the most common of the NCLs, which together, affect nearly 3,000 patients. In pathophysiological terms, interactions between neurons and glial cells play key roles in the emergence and progression of all the NCLs.

Several years of preclinical research directed and funded by BBDF, have led to the discovery and validation of BBDF 101. Like all other drug candidates developed by Theranexus, BBDF 101 is a proprietary combination of drugs relying on the synergistic effect of two approved compounds. In addition to the combination, the agreement also provides for Theranexus to apply its expertise in the pharmacology of neuron-glia interactions to conduct further research on juvenile Batten disease, with the aim of identifying the next generation of treatments for the benefit of the patients.

*"We chose Theranexus as our partner to continue and accelerate the development of BBDF 101 because of their considerable translational expertise in central nervous system disorders, especially in disorders where neuron-glia interactions contribute to the pathophysiology of disease. Beyond the development of BBDF 101, we are also excited that Theranexus will be working alongside us in our research on Batten disease,"* comments Danielle Kerkovich, Ph.D., Principal Scientist at BBDF.

The preclinical research, manufacturing steps, and trial protocol development already performed by BBDF, positions the foundation to apply to the US Food and Drug Administration (FDA) for the Investigational New Drug (IND) status for BBDF 101 which will enable clinical trials to be carried out in the United States.

The trial currently under discussion with the FDA will include safety and pharmacokinetics evaluations as well as assessment of BBDF 101 efficacy as compared with available natural history data. The natural history data is coming from cohorts of patients monitored by University Medical Center Hamburg-Eppendorf, the University of Rochester Batten Center and the National Institutes of Health (NIH). Texas Children's Hospital (TCH), the fourth largest pediatric hospital in the US, located in Houston, Texas will be the primary investigation center for the trial, and Dr. Gary Clark, Chief of Child Neurology and a key opinion leader on NCLs, will be the principal investigator.

*"I am delighted to be able to start work soon on the clinical development of BBDF 101 alongside BBDF and Theranexus. The work done by my fellow scientists at the Jan and Dan Duncan Neurological Research Institute at TCH, which is also part of Baylor College of Medicine, has already demonstrated BBDF 101's potential for juvenile Batten disease. Demonstrating efficacy in patients is the final stage before the drug will be made available to all Batten disease patients, who currently have no therapeutic options,"* comments Gary Clark, Chief of Child Neurology at Texas Children's Hospital.

Success in this trial might lead directly to FDA approval, and the drug is set to benefit from all the regulatory incentives applied for the approval of drugs addressing severe pediatric orphan diseases. Once marketing approval will be granted, Theranexus will lead the marketing process and BBDF will be interested in the additional value creation through milestone payments and royalties on future sales.

*"As a parent and representative for all of the patients and families that we support, who currently have no treatment for this terrible disease, this agreement will help us speed up the development process and tap into Theranexus' resources and expertise to bring BBDF 101 to clinical trials. This is an important partnership and step towards bringing treatment to all of our kids,"* says Craig Benson, Chairman of BBDF and the father of a young patient with Batten disease.

*"We are delighted to be embarking on this partnership with BBDF and we thank them for placing their trust in us. The quality of the preclinical research on BBDF 101 that BBDF has already conducted with the Jan and Dan Duncan Neurological Research Institute at Texas Children's Hospital and Evotec Worldwide, and their collaboration with the NIH Clinical Care Center for the clinical trial, demonstrates the professionalism of their approach and the potential of this drug candidate. We are very proud that Theranexus will have the chance to contribute to the development of a treatment for this fatal childhood disease. This new active ingredient is also a great opportunity to increase the company's value creation potential, as due to its coherence and complementarity with our current developments, the BBDF 101 combination will boost our portfolio of clinical drug candidates, particularly on the US market. This partnership also provides an opportunity to extend our approach based on neuron-glia interactions to lysosomal disorders such as Batten disease."* explains Franck Mouthon, Chairman of Theranexus.

For more information on Theranexus :
https://www.theranexus.com/images/pdf/Investor_presentation_062019.pdf

**About Beyond Batten Disease Foundation**

Beyond Batten Disease Foundation (BBDF) is the world's largest nonprofit organization dedicated to funding research for a treatment and cure for juvenile (CLN3) Batten disease. Since its inception in 2008, over $35 million has been invested in research by leveraging donations, co-funding and strategic partnerships. BBDF is spearheading a unique, cohesive strategy, incorporating independent scientific resources and collaboration with related organizations to drive research in juvenile Batten Disease. Today there is a treatment in sight. BBDF funded research has discovered a drug that slows the progression of the disease in Batten models and is pursuing an FDA approved clinical trial. More information can be found at www.beyondbatten.org

## 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. Theranexus has 3 ongoing clinical development programs: THN 102, currently undergoing Phase II clinical trials, aimed at treating daytime sleepiness in Parkinson's disease patients, THN 201 undergoing Phase Ib trials, designed to treat cognitive disorders in patients with Alzheimer's disease, and THN 101, undergoing Phase Ia trials in patients with neuropathic pain. 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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