PRESS RELEASE

Theranexus

BEYOND BATTEN DISEASE FOUNDATION

Theranexus and Beyond Batten Disease Foundation (BBDF) announce the signing of a worldwide exclusive license for Batten disease drug candidate BBDF-101

- *The agreement covers the development and marketing of the drug following a single clinical trial due to begin in 2020.*
- *If successful, this trial could lead directly to the drug's approval.*

Lyon, 12 December 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 with Beyond Batten Disease Foundation, granting it a worldwide exclusive license to develop and commercialize the drug candidate BBDF-101 for juvenile Batten disease.

This signing of a worlwide exclusive license follows the agreement in principle of June 27th, which granted Theranexus a six-months exclusivity period to finalize a definitive agreement.

Batten disease is a rare, fatal, inherited pediatric disorder of the nervous system for which there is no treatment. It belongs to a group of disorders referred to as neuronal ceroid lipofuscinoses (NCLs). Beyond Batten Disease Foundation (BBDF) funded research aimed at identifying and validating BBDF-101, a proprietary combination of drugs relying on the synergistic effect of two active ingredients, similar to Theranexus' drug candidates currently in clinical development.

The exclusive, global license agreement between BBDF and Theranexus covers the clinical development of drug candidate BBDF-101 to its approval and eventually its commercialization. Theranexus also intends to expand its research on its NeuroLead platform of drug candidates targeting lysosomal disorders associated with neurological symptoms.

"We are delighted to be involved in this partnership with BBDF, enabling Theranexus to extend its approach to lysosomal disorders affecting the nervous system. This asset is a consistent addition to our portfolio, with considerable potential for value creation. We would like to thank BBDF and the patients' families that support it for placing their trust in Theranexus to lead the development and commercialization of BBDF-101 for the benefit of patients," explains **Franck Mouthon, CEO of Theranexus.**

In consideration for this license, the agreement provides for Theranexus to pay BBDF fixed sums on signing, approval, and achievement of commercial objectives post-approval. Moreover, the agreement provides for payment of royalties calculated based on net sales of BBDF-101 once it is marketed by Theranexus.

The clinical trial will begin in 2020 and include efficacy measurements comparing the development of various symptoms to the natural progression of disease previously documented in patient cohorts. The trial will also assess safety and pharmacokinetics. The lead investigation center for the trial will be Texas Children's Hospital (TCH) in Houston, which is the fourth largest pediatric hospital in the United States.

According to initial talks with the FDA, if successful, this pivotal trial alone could be sufficient for approval of this drug candidate in the United States. Once it is approved in the United States, the company intends to apply for the approval of BBDF-101 in Europe on the basis of the same trial.

"As a pediatric neurologist, I am faced with cases of Batten disease in children and teens for whom I sadly have no treatment at present. All the clinical teams are eager to assess the efficacy of BBDF-101 for these patients soon," adds Gary Clark, Professor and Chief of Child Neurology at the Texas Children's Hospital and principal investigator for the clinical trial of BBDF-101. Joining Dr. Clark on the BBDF-101 Advisory Committee are leading experts on juvenile Batten disease including: Jonathan Mink, MD and Erika Augustine, MD at the University of Rochester; Angela Schulz, MD and Miriam Nickel, MD at the University Medical Center Hamburg-Eppendorf; Forbes Porter, MD, PhD, and An Dang Do, MD, PhD, at the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD). NICHD will also serve as the assessment site for the trial. Together, these experts have collected the combined largest natural history dataset in the Batten disease community worldwide.

"Following the success of research sponsored by BBDF to improve understanding of disease mechanisms and identify BBDF-101 as a drug candidate, I am thrilled about this partnership with Theranexus, which will enable the drug's clinical development with a view to finally providing a medical solution offering considerable benefits to children and teens with this disease. We are forever grateful to our donors, volunteers and Batten family partners who made this possible.," concludes Craig Benson, Chairman of BBDF.

About Batten disease

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[1]. In pathophysiological terms, interactions between neurons and glial cells play key roles in the emergence and progression of all the NCLs.

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[2]. 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 the clinical trial of BBDF-101 for Batten disease

The clinical trial will include an adolescent/adult cohort and a pediatric cohort:

- The trial will begin with the enrollment of an adolescent/adult cohort of six patients who will all be administered the drug BBDF-101 in escalating doses, with tolerability and pharmacokinetics established over 5 months. These patients will continue to be administered BBDF-101 throughout the trial and followed up for safety.
- Once measurements of pharmacokinetics and tolerability have been performed for the adolescent/adult cohort, a pediatric cohort of 30 patients will be enrolled in the trial and undergo regular measurements to assess disease progression (vision, cognition, motor symptoms, etc.) over a period of two years.

At the end of the trial, patients' data will be compared to natural disease progression as measured within cohorts already followed up by American and European academic teams.

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[1] National Organization for Rare Disorders (NORD) / Orphanet

[2] Settembre et al, TFEB links autophagy to lysosomal biogenesis, Science 2011

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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