



Theranexus

SHIFTING THE LINES AGAINST
CENTRAL NERVOUS SYSTEM
DISORDERS



BEYOND BATTEN DISEASE
FOUNDATION



THERANEXUS AND BEYOND BATTEN DISEASE FOUNDATION (BBDF) WIN US INVESTIGATIONAL NEW DRUG (IND) APPROVAL TO START CLINICAL DEVELOPMENT OF BBDF 101

Lyon, 9 September 2021 – Theranexus, a biopharmaceutical company innovating in the treatment of neurological diseases, and Beyond Batten Disease Foundation (BBDF), announce receipt of Investigational New Drug (IND) approval from the Food and Drug Administration (FDA) to launch a Phase I/II clinical trial of their drug candidate BBDF 101 for juvenile Batten disease¹, a rare, fatal, genetic disorder.

"With the FDA's IND approval for BBDF 101, we have reached a significant milestone in the drug's regulatory development plan. This approval will enable us to start clinical development of BBDF 101 in Batten disease, for which there is currently no treatment. Our aim is to be able to provide this treatment to patients suffering from Batten disease as soon as possible," explains **Franck Mouthon, CEO of Theranexus**.

In late 2019, Theranexus and BBDF signed an exclusive, global license agreement for the development and commercial use of drug candidate BBDF 101 for Batten disease by the company. BBDF 101, which has received orphan designation in Europe and the United States, is a drug candidate combining trehalose and miglustat, two active ingredients each with its own specific activity of interest for the disease.

The clinical program will start with Phase I/II by the end of 2021. This will involve enrollment of an initial adolescent/adult cohort of six patients who will be administered the drug BBDF 101 as a dose exploration, with tolerability and pharmacokinetics of trehalose, miglustat and a combination of trehalose/miglustat established over 4.5 months. The initial phase of the trial will be followed by a maintenance phase assessing tolerance and efficacy in patients for a period of up to two years during which they will continue to be administered BBDF 101 and be followed up for safety.

Phase III will begin following evaluation of pharmacokinetics and tolerability in Phase I/II and consultation with the FDA. A pediatric cohort of 30 patients will be enrolled in the trial and undergo regular examinations to assess disease progression. The aim will be to compare the development of various symptoms in these patients, as rated on the UBDRS² (neurological and behavioral symptoms, activities of daily living, etc.), over a period of two years, to natural disease progression documented on the basis of patient cohorts already followed up, over a period of two years. The Phase III trial will start in 2022.

The main investigation centers for the trial will be the Texas Children's Hospital (TCH) in Houston, which is the fourth largest pediatric hospital in the United States, the National Institutes of Health (NCIHD³ – Bethesda, Maryland), and the University of Rochester Batten Center (URBC), New York. These institutions are the leading referral centers for Batten disease in the United States. The URBC and NIH have already established cohorts of patients affected by Batten disease to assess the disease's natural history.

¹ Batten disease belongs to a group of disorders referred to as neuronal ceroid lipofuscinoses (NCLs).

² Unified Batten Disease Rating Scale

³ National Institute of Child Health and Human Development (NICHD)

"This approval offers tremendous hope for all patients – children, teens and adults – suffering from Batten disease and their families for whom no medical solution currently exists. We are incredibly grateful for the support of Batten families and our communities in making this important milestone a reality," concludes Craig Benson, Co-Founder and Chair of the BBDF Board of Directors.

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, behavioral 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 patients⁴. 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 Disease⁵. Today there is a treatment in sight. BBDF funded research has discovered a drug that slows the progression of the disease in Batten models. More information can be found at www.beyondbatten.org

Next financial publication:

Thursday 30 September 2021: First half 2021 financial results

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

⁴ National Organization for Rare Disorders (NORD)/Orphanet

⁵ Settembre et al, TFEB links autophagy to lysosomal biogenesis, Science 2011

More information on:

<http://www.theranexus.com>

Click and follow us on Twitter and LinkedIn



Contacts :

THERANEXUS

Thierry LAMBERT

Financial and Administrative Director

investisseurs@theranexus.fr

ACTUS finance & communication

Guillaume LE FLOCH

Investor Relations

+33 (0)1 53 67 36 70

theranexus@actus.fr

FP2COM

Florence PORTEJOIE

Media Relations

+ 33 (0)6 07 76 82 83

fportejoie@fp2com.fr