Theranexus and BBDF granted positive opinion by EMA for the design of Phase III trial to evaluate Batten-1 in CLN3 Batten disease

Lyon, France – Austin, Texas, United States – 7 June 2023 – 7.30 am CEST – Theranexus, a biopharmaceutical company innovating in the treatment of rare neurological diseases, and the Beyond Batten Disease Foundation (BBDF), have today announced receipt of a positive opinion from the European Medicines Agency (EMA) for the design of the pivotal Phase III trial to evaluate their Batten-1 drug candidate for Batten disease.

EMA's response is consistent with the terms of the approval received from the Food and Drug Administration in early May 2023[1] on the design and efficacy endpoints for the Phase III trial to evaluate Batten-1 in CLN3 Batten disease. The same efficacy criteria were selected by EMA, namely visual acuity as the primary endpoint in Phase III and assessment of cognitive and motor function as secondary endpoints.

"*We are delighted to have won EMA approval, following on from initial FDA approval, for the design and endpoints of the pivotal Phase III trial of our Batten-1 drug candidate, to be conducted in Europe and the United States. We are actively preparing to launch the trial by the end of 2023, and are aiming for product approval in these countries followed by the first sales in 2027,*" explained **Theranexus' CEO Mathieu Charvériat.**

"With these two positive concordant opinions, we are delighted to be launching a clinical trial that aims to demonstrate the therapeutic value of Batten-1 for the benefit of all patients, both in America and Europe, as Batten-1 is the only drug candidate in active development with the potential to significantly slow progression of the disease. We are working closely with patient organizations in Europe who, like our American families, are eagerly waiting to enroll in the clinical trial once the European centers open," **concluded Craig Benson, Chair of the Beyond Batten Disease Foundation**.

As a reminder, the Phase III multicenter trial will be a randomized, double-blind study conducted in 2 parallel groups to assess the efficacy of Batten-1 in doses of 15 mg/kg and up to 600 mg/day for a 2-year treatment period, versus placebo. The target population will be a pediatric cohort involving approximately 60 CLN3 patients aged 4 to 16 years, with randomization stratified into 3 age groups of 4 to 8 years, 9 to 12 years and 13 to 16 years to achieve suitable representation for all age groups assessed.

Moreover, an additional cohort of 9 open-label paediatric patients representative of the different ages of the target population will be enrolled in parallel. Interim results will be produced every 6 months on the same efficacy and tolerability endpoints as the main 60 patient cohort, which will enable the company to collect clinical data at an early stage.

Patient enrollment is expected to begin by late 2023. The trial will run in parallel in several centers throughout the United States and Europe.

About Batten-1

Batten-1 is a novel and exclusive proprietary drug containing the active ingredient miglustat. The mechanism of action of this substance blocks the accumulation of glycosphingolipids and neuroinflammation. For patients over 17 years of age in the Phase I/II trial, the product is administered in solid form. In the Phase III trial, it will be administered in a liquid form better suited to pediatric patients.

[1] https://www.theranexus.com/images/pdf/Theranexus_CP_End_of_Phase_2_Meeting_VDEF.pdf

Phase I/II trial design: this is an open-label trial involving 6 patients over 17 years of age with CLN3 Batten disease, treated with miglustat up to 600 mg/day for a 2-year period. The primary endpoint is patient safety and tolerability, assessed using reports of adverse effects, biological tests and ECG, as well as the pharmacokinetics of miglustat. The secondary endpoints include efficacy monitoring: Unified Batten Disease Rating Scale, visual acuity, and brain MRI and optical coherence tomography scans. Administration of Batten-1 in escalating doses with a maximum of 600 mg/day was well tolerated, with no severe side effects observed causing treatment discontinuation. The most commonly reported adverse events are reversible gastrointestinal effects of often light to moderate severity, thus demonstrating the good tolerability profile of Batten-1 in this population. Batten-1 will continue to be assessed in these patients treated over a 24-month period. Further information about the trial is available on https://clinicaltrials.gov/ct2/show/NCT05174039.

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 patients[2]. 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[3]. Today there is a treatment in sight. BBDF funded research has discovered a drug – Batten-1 – that slows the progression of the disease in Batten models. More information can be found at www.beyondbatten.org.

About Theranexus

Theranexus is an innovative biopharmaceutical company that emerged from the French Alternative Energies and Atomic Energy Commission (CEA). The company has a unique platform for the identification and characterization of advanced therapy drug candidates targeting rare neurological disorders and an initial drug candidate in clinical development for Batten disease. Theranexus is listed on the Euronext Growth market in Paris (FR0013286259- ALTHX).

More information at
http://www.theranexus.com
Click and follow us on Twitter and LinkedIn

Contacts

THERANEXUS
Thierry Lambert
Chief Financial Officer
contact@theranexus.com

NEWCAP
Théo Martin/Pierre Laurent
Investor Relations
+33 (0)1 44 71 94 97
theranexus@newcap.eu

FP2COM
Florence Portejoie
Media Relations
+ 33 (0)6 07 76 82 83
fportejoie@fp2com.fr

[2] National Organization for Rare Disorders (NORD)/Orphanet
[3] Settembre et al, TFEB links autophagy to lysosomal biogenesis, Science 2011

Disclaimer

This press release contains certain forward-looking statements concerning Theranexus and its business, including its prospects and product candidate development. Such forward-looking statements are based on assumptions that Theranexus considers to be reasonable. However, there can be no assurance that the estimates contained in such forward-looking statements will be verified, which estimates are subject to numerous risks including the risks set forth in the universal registration document of Theranexus filed with the AMF on 27 April 2023 under number D.23-0345 (a copy of which is available on www.theranexus.com) and to the development of economic conditions, financial markets and the markets in which Theranexus operates. The forward-looking statements contained in this press release are also subject to risks not yet known to Theranexus or not currently considered material by Theranexus. The occurrence of all or part of such risks could cause actual results, financial conditions, performance or achievements of Theranexus to be materially different from such forward-looking statements. Theranexus expressly declines any obligation to update such forward-looking statements.