Theranexus and BBDF confirm positive 18-month results for Batten-1 in Phase I/II trial based on neuronal death biomarker values

Lyon, France – Austin, Texas, United States – 6 June 2024 – 7:30am CT – Theranexus, a biopharmaceutical company innovating in the treatment of rare neurological diseases, and the Beyond Batten Disease Foundation (BBDF), confirm positive results in their Phase I/II trial to evaluate Batten-1 in Batten disease (CLN3) after 18 months of treatment. The results indicate a decline in serum neurofilament light chain (NfL), a biomarker of neuronal death, and confirm the therapeutic potential of the Batten-1 drug candidate in juvenile (CLN3) Batten disease.

Measurements of the concentration of neurofilament light chains (NfL), a recognized biomarker of neurodegeneration, after 18 months of treatment confirm the 12-month results presented at the International NCL2023 Congress in September 2023. This biomarker, measured in the serum of patients compliant to protocol, decreased by an average of 33% after 18 months of treatment (as a reminder, 32% after 12 months of treatment) compared to pre-treatment level.

For Professor Gary Clark, the trial's principal investigator and Chief of Child Neurology at Texas Children's Hospital in Houston "*These results collected after 18 months' treatment are consistent with the absence of marked motor symptom progression reported in these patients. They confirm Batten-1 to be of major interest in the treatment of Batten disease (CLN3) and offer unprecedented prospects for treatment of children with this disease as well as for their families and caregivers".*

For Theranexus Chairman and CEO, Mathieu Charvériat*, "After announcing positive final efficacy and safety data for Batten-1 at the end of April, we are delighted to confirm these positive results today as indicated by the decrease in the levels of a recognized neurodegeneration biomarker. They confirm the 6-month and 12-month outcomes and demonstrate the strong therapeutic potential of Batten 1 in Batten disease. We are continuing to explore all options to ensure we have the necessary resources to implement our pivotal Phase III trial in Europe and the United States".*

Moreover, in collaboration with BBDF and the clinical trial investigators, Theranexus has implemented an Expanded Access Program (EAP) to enable the 6 trial patients to continue receiving the treatment in view of its favorable risk-benefit profile.

The trial results will be presented at the next Translational Research Conference for the Management of NCLs, set to be held from 20 to 22 November 2024 in Chicago.

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, thus significantly reducing neuronal death that contributes to a progressive loss of function in patients. 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.

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 an 18-month 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 biomarkers (NfL, glycosphingolipds), efficacy monitoring: Unified Batten Disease Rating Scale, visual acuity, measurement of brain volumes by MRI and measurement of the thickness of the neuronal layer of the retina by 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. 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 and is followed by cognitive disorders, behavioral disorders, and motor disorders. 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.

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 2,000 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. 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).

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

For more information:
http://www.theranexus.com
Follow us on Twitter and LinkedIn :

Contacts:

THERANEXUS
Christine PLACET
Chief Financial Officer
contact@theranexus.com

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

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