Highly Promising 12-month results in the Phase I/II trial of Batten-1

Presentation at the International Congress on Neuronal Ceroid Lipofuscinoses (Batten Disease) NCL2023

Lyon, France – Austin, Texas, United States – 29 September 2023 – 7.30am – CET – Theranexus, a biopharmaceutical company innovating in the treatment of rare neurological diseases, and the Beyond Batten Disease Foundation (BBDF), present very encouraging interim results for efficacy and safety after 12 months of treatment in their Phase I/II trial for Batten disease (CLN3) at NCL2023, the International Congress on Neuronal Ceroid Lipofuscinoses held in Hamburg (Germany) from 26 to 30 September 2023.

For Professor Gary Clark, the trial's principal investigator and Chief of Child Neurology at Texas Children's Hospital in Houston, "*The Phase I/II results for Batten-1 are highly promising, as we have observed a reduction in neurofilaments and glycosphingolipids and no notable progression of motor symptoms after 12 months of treatment in our 6 patients aged 17 years and over. This is unprecedented in the indication, and Batten-1 is thus a source of great hope for children affected by the disease and for their families*". **Professor Clark will be presenting the 12-month interim efficacy and safety results at the "Translational Research Clinical" session on Friday 29 September at 12.25pm (CEST).**

The 12-month treatment results further support the 6-month results announced in mid-June 2023. **The new results, achieved after 12 months of treatment, show an average 32% decline in neurofilament light chain (NfL) levels in patient serum (as a reminder, 17% after 6 months' treatment). Neurofilaments are a recognized biomarker of neurodegeneration[1]. A 64% reduction in this biomarker is also observed in the cerebrospinal fluid (CSF).** After twelve months of treatment, in line with preclinical data the results also confirmed a reduction in the glycosphingolipids involved in the disease (Gb3 reduction of 45%) which, when they accumulate, are toxic to neurons. Clinically, there was notably less worsening of motor symptoms evaluated by the modified UBDRS physical assessment subscale after 12 months than what is expected in this naturally progressive disease.

For Theranexus' CEO, Mathieu Charvériat: *"We are delighted with these 12-month results which confirm and complement our initial 6-month results. The decrease of biomarker levels confirms the effect of Batten-1 on neuronal death, and the clinical course did not worsen. These consistent results represent a significant endorsement of the mechanism of action and demonstrate the strong therapeutic potential of Batten-1 in Batten disease (CLN3)".*

As a reminder, the Phase I/II trial conducted by Theranexus and BBDF to evaluate their drug candidate, Batten-1, includes six patients with Batten disease (CLN3) aged 17 years and over. The patients are being treated over a 24-month period.

The presentation of the 12-month efficacy and safety results will be available online on 29 September after market closing.

For further information about NCL2023: https://ncl2023.de/

Upcoming publications:

Detailed slides describing the 12-month results available on the company website (https://www.theranexus.com/fr/actionnaires/documents.html): 29 September 2023 after market closing.

17 October 2023: Cash position as of 30 September 2023

[1] Dang Do AN, et al. Neurofilament light chain levels correlate with clinical measures in CLN3 disease. Genet Med. 2021 Apr;23(4):751-757

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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 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 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. 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 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[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.

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

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).