THERANEXUS AND BEYOND BATTEN DISEASE FOUNDATION ANNOUNCE STRONG POSITIVE REAL-WORLD DATA SUPPORTING BATTEN-1 EFFICACY FOR THE TREATMENT OF BATTEN DISEASE

Lyon, France – Austin, Texas, United States – Mai 13, 2025 – 6.00 pm CET – Theranexus, a biopharmaceutical company developing drug candidates for rare neurological diseases and the Beyond Batten Disease Foundation (BBDF), today announced new real-world data strongly supporting the efficacy of Batten-1 (miglustat) as a treatment for CLN3 disease, also known as juvenile Batten disease.

The analysis evaluated visual acuity outcomes in CLN3 patients treated with Batten-1 (miglustat, n=11), versus untreated patients (n=22), using real-world data collected from natural history studies, family interviews, published reports, and prescribers' personal communications. This analysis focused on patients with measurable visual acuity at baseline (visual acuity score ≤ 1.9 LogMAR) and compared the progression of visual function between groups over a 12-month period. To ensure an appropriate comparison between the treated and untreated groups, a propensity score methodology was applied.

The analysis demonstrated a **statistically significant and clinically meaningful difference in favor of Batten-1 treatment on visual function with a notable preservation of visual acuity in treated patients compared to untreated patients.** The notable preservation of visual acuity in treated patients compared to untreated patients add to the positive results from the Phase 1/2 study of Batten-1[1] and underscore the potential of miglustat to address critical unmet needs in Batten disease. The visual acuity endpoint used in the real-world analysis is the same as that selected for the planned pivotal Phase 3 clinical trial of Batten-1, a design endorsed by the U.S. Food and Drug Administration (FDA)[2] and the European Medicines Agency (EMA)[3].

"*We are deeply grateful to the patients, their families, and the healthcare providers who made this important analysis possible by agreeing to share their experiences and those unique clinical data. This collaboration has been essential in demonstrating Batten-1 potential to change the course of CLN3 disease*" explains **Craig Benson, Chairman of the Beyond Batten Disease Foundation**.

"*Achieving near stabilization of visual acuity over 12 months in CLN3 patients is truly remarkable and unprecedented. In this population, we would typically expect a relentless decline in vision. These results offer real hope for altering the natural progression of the disease*," commented **Dr. Gary Clark, MD, Chief of Neurology and Developmental Neuroscience at Baylor College of Medicine, Houston, Texas**.

"*These results have been observed using the primary efficacy endpoint defined in our Phase 3 trial, this is highly reassuring. These findings strongly support our development strategy and reinforce the relevance of our endpoint in demonstrating Batten-1 potential efficacy*" concluded **Marie Sebille, Chief Medical Officer at Theranexus**.

Results will be submitted for presentation at the NCL 2025 Conference, to be held in Australia in October.

[1] https://www.theranexus.com/images/pdf/Theranexus_PR_Results_18_Nf_biomarkers_DEF.pdf
[2] https://www.theranexus.com/images/pdf/Theranexus_PR_End_of_phase_2_meeting_VDEF1.pdf
[3] https://www.theranexus.com/images/pdf/Theranexus_PR_Positive_Opinion_EMA_VDEF.pdf

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[4]. 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).

For more information:
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[4] National Organization for Rare Disorders (NORD)/Orphanet

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