# Theranexus announces positive data on efficacy and safety in the Phase I/II trial of Batten-1

## *Stabilization of motor symptom progression in young adult patients suffering from juvenile Batten disease (CLN3) after 18 months of treatment*

**Lyon, France – Austin, Texas, United States – 17 April 2024 – 3pm CET** – Theranexus, a biopharmaceutical company innovating in the treatment of rare neurological diseases and the Beyond Batten Disease Foundation (BBDF), today announce final positive data on efficacy and safety in the Phase I/II trial of its drug candidate Batten-1 in juvenile Batten disease (CLN3) after 18 months of treatment.

The 18-month safety and efficacy data confirm the 12-month results announced in September 2023. Batten-1 presented a good safety profile. On average, in the 6 young adult patients, treated with Batten-1, progression of motor symptoms was considerably slowed down and appeared stable compared to progression in untreated patients, as evaluated by the modified UBDRS Physical Assessment score. The mean change from baseline was +1,83 for the six subjects treated over 18 months vs +6,04 in untreated subjects from the natural history study conducted by the University of Rochester (n=46).

**For Professor Gary Clark, the trial's principal investigator and Chief of Child Neurology at Texas Children's Hospital in Houston**, *"The data collected after 18 months of treatment with Batten-1 further reinforce its highly promising potential. We currently no longer observe a marked progression of motor symptoms in the 6 patients treated. These results support the prospect of a major benefit of Batten-1 for the children with this very severe disease and for their families"*.

**For Theranexus' CEO, Mathieu Charvériat**: "*These positive results on efficacy and safety of Batten-1 are highly encouraging for the patients and their families. Together with BBDF and the investigators we are initiating an Expanded Access Program (EAP) to enable the 6 patients previously in the trial to continue receiving the treatment, considering its favorable risk-benefit profile. These results highlight the strong therapeutic potential Batten-1 on the clinical course of the disease. In this context, we are exploring different funding options to ensure we have the adequate resources to launch a phase 3 pivotal trial, which design has already received positive opinions by the FDA and the EMA*".

As a reminder, the Phase I/II trial conducted by Theranexus and BBDF to evaluate their drug candidate, Batten-1, included six patients with juvenile Batten disease (CLN3) aged 17 years and over. The patients were treated for 18 months. Following their participation in the trial, all patients are offered the possibility to continue receiving the treatment through a compassionate use program.

## 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

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