

Update on the Theranexus' Batten-1 project in CLN3 Batten's disease

- A communication by BBDF and Theranexus of the promising results of the Batten-1 Phase I/II trial at the Child Neurology Society Annual Meeting in San Diego (California)
- Additional expanded use of miglustat providing positive signals on visual acuity in younger CLN3 patients

Lyon, France – Austin, Texas, United States – November 19, 2024 – 6 PM CET – Theranexus, a biopharmaceutical company innovating in the treatment of rare neurological diseases, and the Beyond Batten Disease Foundation (BBDF) presented the final and positive results of the Phase I/II trial to evaluate Batten-1 in 6 young adult patients with CLN3 Batten's disease after 18 months of treatment, at the Child Neurology Society Annual Meeting in San Diego, 11 to 14th November.

This presentation highlights the strong safety profile of miglustat in CLN3 Batten disease patients and underscores the positive efficacy data gathered during this trial. The findings demonstrate clear target engagement, a biological effect, and are suggestive of a stabilization of disease progression over the treatment period. All trial participants chose to continue receiving miglustat through the BBDF's early access program, with no safety concerns reported after two years of ongoing treatment.

Additionally, case reports from expanded use of miglustat in CLN3 Batten disease further support its potential as a disease-modifying therapy. These reports suggest a possible stabilization of visual acuity—a function critically impacted in CLN3 Batten disease, often leading to legal blindness by age 12. This information is encouraging, especially in light of discussions between Theranexus, BBDF, the FDA, and EMA on using visual acuity as the primary endpoint for the upcoming Phase III trial, aimed at supporting the drug's registration in the U.S. and Europe.

The clinicians who treated these patients as well as other younger patients with miglustat, are enthusiastic about these promising data. **For Professor Gary Clark, the trial's principal investigator and Chief of Child Neurology at Texas Children's Hospital in Houston** *“these case reports on visual acuity, in conjunction with other personal positive observations on visual experience in younger patients, complement our Phase I/II trial results that support the prospective benefit of Batten-1 in the treatment of CLN3 Batten's disease. Upon confirmation in a larger trial, these results with Batten-1 will be a breakthrough in the management of CLN3 Batten disease.”*

Mathieu Charvériat, CEO and Chairman of Theranexus concludes: *“Our positive Phase 1/2 study, as well as very promising additional case reports of expanded use of miglustat with positive outcome on vision, strongly support the launch of our phase 3, as well as the selection as visual acuity as primary endpoint. As indicated before, we explore options to have the necessary resources for our pivotal Phase III trial, and notably through the future commercialization of our liquid formulation in Niemann-Pick Type C as recently announced.”*

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:

<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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