THERANEXUS, BBDF AND CARDIFF UNIVERSITY PRESENT THEIR NOVEL RESEARCH ON BATTEN-1 AND BATTEN DISEASE AT WORLDSYMPOSIUM 2023

From 22 to 26 February 2023 in Orlando, Florida

- **Presentation of preclinical data demonstrating how Batten-1 drastically reduces the buildup of toxic glycosphingolipids in Batten disease and a poster describing for the first time the burden of Batten disease.**

Lyon, France – Austin, Texas, United States – Cardiff, UK – 20 February 2023 – 6pm CET – Theranexus, a biopharmaceutical company innovating in the treatment of neurological diseases and pioneer in the development of drug candidates modulating the interaction between neurons and glial cells, and its partners Beyond Batten Disease Foundation (BBDF) and Cardiff University, today announced several presentations at WORLDSymposium 2023, an international scientific event focused on lysosomal diseases (22 to 26 February in Orlando, Florida – United States).

At the conference, the research conducted by Cardiff University, Theranexus and BBDF will be showcased in five presentations, including one oral presentation.

- An oral session entitled "**The Batten disease associated protein CLN3 is required for the efflux of lysosomal potassium**", presented by Dr. Hannah Best of Cardiff University, will feature her research on the CLN3 protein in Batten disease on Wednesday 22 February at 9am. Dr. Best will receive the Young Investigator Award for her outstanding research.
- The session will be followed by a poster session presenting our novel therapeutic strategy that uses Batten-1 to treat Batten disease by reducing toxic glycosphingolipids in neurons (Poster 229, **Glycosphingolipid reduction with miglustat as a therapeutic strategy for CLN3 and other neuronal ceroid lipofuscinoses**, Dr. Emyr Lloyd-Evans, Cardiff University).
- A broader presentation of the effects of Batten-1 active ingredient, miglustat, will also be given on the same day (Poster 230, **Deciphering the various mechanisms of action of miglustat in the lysosomal disorders**, Drs Emyr Lloyd-Evans and Charles Evans, Cardiff University).
- Lastly, a new series of studies will be presented, highlighting for the first time the burden of the disease as experienced by patients and their families (Poster 185, **Understanding the functional burden of CLN3 through the eyes of patients and families**, Skyler Jackson, Engage Health, BBDF, Theranexus).

Theranexus CEO, Mathieu Charvériat explains: *"These different presentations by internationally recognized teams confirm the solid scientific and clinical rationale underpinning the development of our asset Batten-1 in juvenile Batten disease, for which there are significant unmet medical needs representing a considerable burden for families. To our knowledge, Batten-1 is the only candidate in active clinical development to tackle the major medical challenges of this disease. In this context, we are currently preparing the Phase III trial which should begin during 2023."*

For Dr. Emyr Lloyd-Evans, an expert in lysosomal disease indications from Cardiff University, *"We are delighted to present our innovative research on CLN3 and miglustat to our peers, underlining a solid rationale for the use of Batten-1 in juvenile Batten disease. In this disease, as in the other two similar indications for which miglustat is registered and efficacious in patients, the drug blocks the toxic buildup of glycosphingolipids. Our scientific data and in-depth knowledge of miglustat therefore converge towards a potential efficacy of this drug candidate in Batten disease and we are excited for it to be tested in the Phase III trial to be conducted by Theranexus and BBDF."*

"We present initial research quantifying the significant burden suffered by patients, families and caregivers, which justifies the absolute need to develop novel therapeutic approaches. The research also highlights, as regularly observed in our discussions with patients' families and advocacy organizations, the wish of families to take part in clinical developments such as those led with Theranexus," **concludes Craig Benson, Chair of Beyond Batten Disease Foundation**.

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. 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 efficacy monitoring: Unified Batten Disease Rating Scale, visual acuity, and brain MRI and 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 which is followed by personality changes, behavioral problems, and slowed learning. 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. Psychiatric symptoms or psychosis can appear at any time.

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 3,000 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[2]. 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). It specializes in the treatment of central nervous system disorders and is a pioneer in the development of drug candidates targeting both neurons and glial cells.
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
[2] Settembre et al, TFEB links autophagy to lysosomal biogenesis, Science 2011

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