New advance to the Theranexus and BBDF Batten-1 program for juvenile Batten disease (CLN3)

Lyon, France – Austin, Texas, United States – 5 January 2023 – 6.00 pm 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 the Beyond Batten Disease Foundation (BBDF), have today announced the initial results of their Phase I/II clinical trial for juvenile Batten disease (CLN3).

After a 9-week course of Batten-1 with the maximum dose of 600 mg/day, the first results of the Phase I/II trial enrolling six patients with juvenile Batten disease (CLN3) aged 17 and over showed good patient safety and tolerability, and a pharmacokinetic profile in line with expectations.

Craig Benson, Chair of the Beyond Batten Disease Foundation, explained: "*We are delighted that Batten-1 showed good safety and tolerability for our patients suffering from Batten disease. The trial results are highly encouraging and offer tremendous hope for all those families living with this neurodegenerative disease for which there is currently no available treatment.*"

In conclusion, Theranexus' Chief Medical Officer Marie Sebille added: "*We have achieved an important milestone with these Phase I/II results in CLN3 patients, enabling us to prepare the protocol of the Phase III trial to launch our pivotal clinical trial involving a pediatric cohort during 2023.*"

As a reminder, for the Sachs Associates 6th Annual Neuroscience Innovation Forum, to be held in San Francisco on 8 January 2023, Theranexus[1] will be presenting its latest advances on the Batten-1 program and also be participating in a roundtable discussion on the theme "*Rare and orphan diseases new modalities panel.*" In addition, the company will be exhibiting at Biotech Showcase and at the same time participating in the 41st annual J.P. Morgan Healthcare Conference bringing together key healthcare leaders.

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.

[1] https://www.theranexus.com/images/pdf/Theranexus_CP_Conference_JP_Morgan_FR.pdf

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[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.

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).

Next financial publication:

18 January 2023: Cash position as at December 31th 2021

More information at
http://www.theranexus.com
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[2] National Organization for Rare Disorders (NORD)/Orphanet
[3] Settembre et al, TFEB links autophagy to lysosomal biogenesis, Science 2011