THERANEXUS AND BBDF PRESENT PROGRESS ON THE BATTEN-1 PROGRAM AT THE TRANSLATIONAL RESEARCH CONFERENCE FOR THE MANAGEMENT OF NCLS ON 3 AND 4 NOVEMBER, 2022 IN CHICAGO

Lyon, France – Austin, Texas, United States – 2 November 2022 – 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 the Beyond Batten Disease Foundation (BBDF), have today announced the presentation of research, supported by BBDF, from Dr. Emyr Lloyd-Evans of Cardiff University's School of Biosciences, at the 7th edition of the Translational Research Conference for the Management of NCLS to be held on 3 and 4 November 2022 in Chicago (United States).

The presentation, entitled "**How glycosphingolipid accumulation impacts the pathophysiology of NCLs: development of substrate reduction therapy with Batten-1 for treating CLN3 disease and other NCLs**," will review new preclinical data generated with relevant disease models and showing the efficacy of Batten-1, focusing on both improved understanding of the biology of Batten disease and the mechanism of action of Batten-1's active substance, miglustat. The research was conducted by the team led by Dr. Emyr Lloyd-Evans, an expert in the pathophysiology of NCL-type lysosomal disease indications and the mechanism of action of miglustat for these indications.

The 7th edition of the Translational Research Conference for the Management of NCLS brings together the world's best preclinical teams in the field of NCLs which include Batten disease (CLN3), in association with patient organizations, clinicians and field experts, as well as industry stakeholders involved in clinical developments in this group of indications.

Dr. Emyr Lloyd-Evans of the Cardiff University School of Biosciences, explains: "*Batten disease is caused by a mutation of the CLN3 gene; our recent research, together with that of other internationally renowned teams, indicates that this mutation produces an accumulation of complex molecules called glycosphingolipids which cause neuronal death. I am delighted to present our results and demonstrate how miglustat is capable of reducing the accumulation of toxic products in the cell, the aim being to prevent patients' conditions from worsening*."

Mathieu Charvériat, Co-founder, Deputy CEO and Chief Scientific Officer of Theranexus, comments: *"This new communication is an opportunity for Theranexus to strengthen ties with world experts on these diseases and highlight progress on our drug candidate Batten-1. In this regard, the tolerability and pharmacokinetic data for Batten-1 that we are currently collecting should enable us to begin the Phase 3 trial in 2023. Our therapeutic goal with this first treatment for Batten disease is to alter the disease course*".

About Batten-1

Batten-1 is a novel and exclusive proprietary drug containing the active ingredient miglustat. The mechanism of action of this substance substantially reduces the accumulation of glycosphingolipids, that causes neuroinflammation and also contributes to neuronal excitotoxicity, to prevent brain cell death. In the Phase I/II trial, Batten-1 is administered to patients over 17 years of age in solid form. In Phase III, it will be administered in a liquid form better suited to children.

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).

Next financial publication:

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

[1] National Organization for Rare Disorders (NORD)/Orphanet
[2] Settembre et al, TFEB links autophagy to lysosomal biogenesis, Science 2011

More information at http://www.theranexus.com
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