PRESS RELEASE

Theranexus

SHIFTING THE LINES AGAINST CENTRAL NERVOUS SYSTEM DISORDERS

BEYOND BATTEN DISEASE FOUNDATION

THERANEXUS AND BBDF START RECRUITMENT FOR PHASE I/II TRIAL IN BATTEN DISEASE PATIENTS

Lyon, France – Austin, TX, USA - 3 February 2022 – 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), are starting recruitment for Phase I/II clinical trial to evaluate BBDF-101 in Batten disease.

Franck Mouthon, Chairman of Theranexus, said: *"The launch of recruitment is a key step in the development of our drug candidate BBDF-101. This trial will be conducted with BBDF and in collaboration with leading North American referral centers for Batten Disease, a rare, fatal, genetic disorder of the nervous system. Our ambition is to demonstrate the full potential of BBDF-101 in this disease for which there are currently no therapeutic options for patients."*

Following on from the award of Investigational New Drug (IND) approval from the Food and Drug Administration (FDA) for its BBDF-101 program in 2021, Theranexus is now actively recruiting patients for its Phase I/II clinical trial. During the 2-year trial, six patients 17-year-old or older will receive BBDF-101 in escalating doses during the first 4.5 months to establish tolerability and pharmacokinetics of trehalose, miglustat and a combination of trehalose/miglustat. The results of the titration phase will be available at the start of the second half of 2022.

After consulting with the FDA, at the end of 2022 Theranexus plans to start a pivotal Phase III trial involving a pediatric cohort of 30 patients with regular measurements to assess disease progression and safety. The aim will be to compare the development of various signs and symptoms in these patients, as rated on the Unified Batten Disease Rating Scale (UBDRS, neurological and behavioral symptoms, activities of daily living, etc.), over a period of two years, to data from a study of disease progression in a cohort of untreated patients followed for many years at the University of Rochester.

This Phase III trial will be conducted in collaboration with the leading referral centers for Batten disease in the United States which include the Texas Children's Hospital in Houston, TX, the fourth largest pediatric hospital in the United States, the National Institutes of Health, and the University of Rochester Batten Center in Rochester, NY.

"It is with great gratitude for our patients' families and donors that we now see the launch of the BBDF-101 clinical program which aims to offer a therapeutic solution for patients suffering from this very rare genetic disease. Achieving this major milestone for the Foundation, in collaboration with Theranexus, is an exciting culmination of almost 12 years of research," concludes **Craig Benson, Chair of the BBDF Board of Directors.**

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 that slows the progression of the disease in Batten models. More information can be found at www.beyondbatten.org

About development of BBDF-101 for Batten disease and the partnership between BBDF and Theranexus

The research funded by BBDF and conducted by academics specializing in lysosomal storage disorders (particularly Baylor College of Medicine) led to the discovery of the drug candidate BBDF-101. Following a partnership agreement between BBDF and Theranexus concluded in late 2019 (see below), Theranexus supplemented preclinical development of BBDF-101 with research to establish product safety. Thanks to the research findings, BBDF-101 will now be able to enter the clinical phase following the grant of IND status by the FDA in September 2021.

The clinical development pathway includes:

- Phase I/II for which recruitment began today,
- Phase III due to start by the end of 2022 and to include 30 patients with the aim of achieving final results in the second half of 2025,

The development of BBDF-101 is the result of a partnership concluded between BBDF and Theranexus in late 2019. The exclusive, global license agreement between BBDF and Theranexus covers the clinical development of drug candidate BBDF-101 pending approval and its commercial use. Theranexus also plans to continue its research on the NeuroLead platform of drug candidates targeting neurological forms of other lysosomal disorders.

In consideration for this license, the agreement provides for Theranexus to pay BBDF fixed sums on signing, approval, and achievement of commercial objectives post-approval. Moreover, the agreement provides for payment of royalties calculated based on net sales of BBDF-101 once it is marketed by Theranexus

Next financial publication:

Thursday 28 April 2022: 2021 annual financial results and update on cash position on 31 March 2022

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

ABOUT THERANEXUS

Theranexus is a clinical-stage biopharmaceutical company that emerged from the French Alternative Energies and Atomic Energy Commission (CEA) in 2013. It develops drug candidates for the treatment of nervous system diseases.

Thanks to its knowledge of neuron and glial cell interactions, THERANEXUS is a pioneer in the design and combination of approved substances and has a solid and diversified portfolio of drug candidates in clinical-phase testing. The company's combined drug repurposing strategy based on a solid commercial footing and a capability to rapidly demonstrate its clinical worth, enables it to produce different high-value-added proprietary drug candidates, significantly reduce development time and costs, and considerably increase the chance of its drugs reaching the market.

Accordingly, THERANEXUS is well-positioned in several indications, including for Parkinson's and Batten disease, for which there is currently no treatment available.

Theranexus is listed on the Euronext Growth market in Paris (FR0013286259- ALTHX).

More information on:
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