

THERANEXUS UNVEILS ITS SCIENTIFIC ADVANCES ON THE OCCASION OF WORLD RARE DISEASE DAY 2022

- *New milestone for NeuroLead, the active agent screening platform*
 - *New publications in scientific journals*
 - *Start of Phase I/II clinical trial for Batten disease*

Lyon, 28 February 2022 – On the occasion of World Rare Disease Day 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, takes the opportunity to report on its latest scientific developments with new advances on its active agent screening platform NeuroLead in rare neurological disorders in collaboration with the Collège de France and the French Alternative Energies and Atomic Energy Commission (CEA), and new academic publications.

NeuroLead, the platform for generating new drug candidates for innovative therapies in orphan neurological indications

Theranexus, a spin-off of the CEA, which coordinates the NeuroLead program, in collaboration with the Collège de France and the CEA, recently completed the first scientific phase of the platform, based on the implementation and validation of models of interactions between neurons and astrocytes and the study of their functions. These models will serve as a basis for identifying and characterizing new drug candidates in orphan neurological indications. With the achievement of this new milestone, in February 2022 Theranexus received a payment of €1.7 million from Bpifrance through the Investments for the Future program (PIA). In addition to this sum, funding of €800,000 was also provided by a Bpifrance R&D Innovation Loan to speed up development of the platform.

As a reminder, in 2019 Theranexus and its partners secured total funding of €6.2 million through the PIA program. Of the €4 million granted to Theranexus, the Company has received a total of €2.7 million to date.

"We are very pleased to have successfully achieved this initial scientific phase which will enable us to proceed with the identification and development of new drug candidates in rare nervous system diseases, and to expand our portfolio in this area beyond our asset BBDF-101 for Batten disease. We are delighted with the new prospects afforded by the NeuroLead platform", stated **Mathieu Charvériat, Deputy CEO and Chief Scientific Officer of Theranexus.**

"With the NeuroLead platform we now have new preclinical models to better target interactions between neurons and astrocytes in a physiological setting, and we are currently working on rare disease models", continued **Dr. Nathalie Rouach from the Center for Interdisciplinary Research in Biology at the Collège de France.**

"We are working on the co-culture of human induced pluripotent stem cells, differentiated into neurons and astrocytes, which constitutes human tissue that is ideal for identifying new drugs in orphan neurological diseases", concluded **Dr. Anselme Perrier from the François Jacob Institute of Biology, Molecular Imaging Research Center (MIR Cen) at the CEA.**

Three new publications in scientific journals

In January 2022, Theranexus, the Collège de France and the CEA published a state of knowledge report on the interactions between astrocytes and neurons in orphan indications and their modeling through the use of stem cells in the **International Journal of Molecular Sciences**, entitled "[Modeling and Targeting Neuroglial Interactions with Human Pluripotent Stem Cell Models](#)".

In December 2021, Theranexus published another article entitled "[Automated Assays to Identify Modulators of Transcription Factor EB Translocation and Autophagy](#)" in **ASSAY and Drug Development Technologies**, presenting its research on the identification of new molecules in the treatment of lysosomal storage disorders, a group of 50 orphan conditions that affect more than 3,000 adults and children in France.

Theranexus also recently presented its new neuroimaging research, with the first ever demonstration of functional ultrasound imaging for the characterization of disease models, paving the way for the use of such models in orphan diseases. The article entitled "[Impaired local and long-range brain connectivity and visual response in a genetic rat model of hyperactivity revealed by functional ultrasound](#)" was published in **Frontiers in Neuroscience** in February 2022.

Start of Phase I/II clinical trial for Batten disease, a rare degenerative genetic disorder

Lastly, in early February 2022 Theranexus and BBDF started recruitment for a Phase I/II clinical trial to evaluate BBDF-101 in Batten disease, a rare and fatal genetic nervous system disorder for which there is currently no treatment or cure. The initial results of the titration phase will be available at the start of the second half of 2022 and, after consulting with the FDA, at the end of 2022 Theranexus aims to start pivotal Phase III trials.

About the NeuroLead platform

In early 2019, the NeuroLead platform was awarded a €6.2 M funding package through the Investments for the Future program (PIA), managed by Bpifrance (Structuring Research and Development Projects for Competitiveness, PSPC) to support its development. The NeuroLead program, spanning four years, is coordinated by Theranexus in collaboration with the Collège de France and the French Alternative Energies and Atomic Energy Commission (CEA). Combining the latest innovations in neuroscience and artificial intelligence, the program aims to design and industrialize a unique drug candidate identification and characterization platform targeting interactions between the two major brain cell populations, neurons and glial cells, for treating neurological disorders.

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 Batten disease, for which there is currently no treatment available. Theranexus is listed on the Euronext Growth market in Paris (FR0013286259- ALTX).

About the Collège de France

The Collège de France is a public higher education institution that is unique in France and has no equivalent abroad. Since the sixteenth century, the Collège de France has fulfilled a dual role: to conduct the most ground-breaking research and to teach this. Dedicated to fundamental research, the Collège de France has the unique mission of "teaching knowledge in the making in every field of literature, the arts and sciences". It works in partnership with the National Centre for Scientific Research (CNRS), the French National Institute of Health and Medical Research (INSERM) and several other leading institutions. The Collège de France's Center for Interdisciplinary Research in Biology (CIRB), a specific partner of the Neurolead program, occupies a prominent position across critical themes and research priorities, acting under the direction of Marie-Hélène Verlhac. Nathalie Rouach's team, actively committed to Neurolead, is an internationally acclaimed authority on such cellular interactions.

About the CEA et MIRCEN

The CEA is a major player in research, serving the State, the economy and citizens. It provides concrete solutions to their needs in four main areas: energy transition, digital transition, technologies for the medicine of the future, defense and security. The CEA/MIRCen is one department within the François Jacob Institute of Biology of CEA headed by Dr. Philippe Hantraye, a member of the Neurolead project. This research facility, the only one of its kind in Europe, hosts researchers from a variety of organizations and carries out translational research on neurodegenerative diseases and develops innovative therapies in an optimal scientific, technological and methodological setting. The primary objective is to enhance the transfer of knowledge and technology from basic neuroscience research to clinical trials in patients with neurodegenerative disorders. MIRCen also oversees a National Infrastructure in Biology and Health (INBS), NeurATRIS, involving prestigious research centers in the Paris region.

Next financial publication:

Monday 25 April 2022: 2021 annual financial results and update on cash position on 31 March 2022

More information on:

<http://www.theranexus.com>

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**Contacts****THERANEXUS**

Thierry LAMBERT

Chief Financial Officer

contact@theranexus.com

NEWCAP

Théo MARTIN/Pierre LAURENT

Investors Relations

+33 (0)1 44 71 94 97

theranexus@newcap.eu

FP2COM

Florence PORTEJOIE

Media Relations

+ 33 (0)6 07 76 82 83

fportejoie@fp2com.fr

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