



Shareholders' Letter

Lyon, France – January 9, 2025

Dear Shareholders,

At the beginning of this year 2025, I would like to warmly thank you for your trust and constant commitment to Theranexus. The year 2024 was marked by significant advances as the result of our shared vision and the unwavering dedication of our team. I would like to summarize them today and share with you our outlook for 2025.

A first industrial partnership in two rare neurological diseases for the TX01 drug

Our industrial partnership with the Insud Pharma / Exeltis group is a major milestone in the growth of our company. It aims to commercialize our drug candidate TX01 in two neurological diseases, Niemann-Pick type C disease and Gaucher disease. This innovative new formulation meets the needs of approximately 2,000 patients worldwide and addresses a market of nearly €200 million. This agreement illustrates our ability to design and develop drug candidates with high medical and economic value. This first validation by a global industrial player and the preparation for the market entry of our first drug is a major achievement and success in Theranexus' journey as a biopharmaceutical company.

The development of TX01 is fully funded by the €2 million advance payment granted by Insud Pharma / Exeltis as part of our license agreement. The marketing authorization application in Europe is expected to be submitted in the second half of 2025, with other territories to follow later.

Major advances in the development of our Batten-1 drug candidate for Batten disease

Theranexus, in partnership with the Beyond Batten Disease Foundation (BBDF), is developing the Batten-1 drug candidate for the juvenile form of Batten disease (known as "CLN3"). In 2024, we demonstrated and published the solid safety profile of Batten-1 in patients with CLN3 and highlighted the positive efficacy data collected during this phase 1/2 trial. The results demonstrate clear target engagement, a sound biological effect on neuronal cell death, and suggest stabilization of clinical progression during the treatment period. Additionally, case reports reinforce its potential as a first disease-modifying treatment of Batten disease and suggest possible stabilization of visual acuity, a function severely impacted in Batten CLN3 disease. We have also validated our phase 3 study plan with both the American (FDA) and European (EMA) regulatory authorities.

This phase 3 requires €10 million in funding. We continue to explore all possible avenues, including an industrial partnering agreement, to launch this study in the course of 2025.

A new drug candidate from our antisense oligonucleotide platform

The company has identified, optimized, and patented a new antisense oligonucleotide, an innovative drug candidate targeting TFEB, a protein recognized as controlling autophagy, for patients with rare neurological diseases, particularly lysosomal diseases. With this project, the company, along with its two partners INSERM and Diverchim, has formed the PickASO consortium, which is supported by a total funding of €4.7 million from Bpifrance to develop its drug candidate up to its entry into clinical development. The drug candidate has also received financial support for €320,000 from the Auvergne-Rhône-Alpes Region, in collaboration with the Lyon Neuroscience Research Center and CERMEP.

Strengthening our financial position

The company has continued to manage its expenses rigorously and has set up an equity line with our partner financial partner Iris. Additionally, we have received support from all our banking partners, including Bpifrance, which recently granted a 21-month deferral of the company's bank debt repayment. These measures, combined with the successful execution of the contract with Exeltis, ensure our financial visibility until the first quarter of 2026.

These results for 2024 and perspectives for 2025 rely on our ability to innovate, the consistency of our scientific approach, and the unwavering support of our partners and shareholders.

On behalf of our team, I thank you for your trust and support in this fantastic journey. Together, we will continue to build a promising future for Theranexus.

With all my consideration,

Mathieu Charvériat

Chairman and CEO of Theranexus