Theranexus Becomes THX Pharma: A Strategic Evolution Toward the Commercialization of Its Medicines for Rare Diseases

With TX01 and Batten-1, THX Pharma is entering a new strategic phase focused on regulatory approval, early access, and international commercialization of its medicines.

Lyon, France – September 29, 2025, 6.30 pm CEST – THX Pharma (formerly Theranexus), a pharmaceutical company specializing in rare neurological diseases, today unveils its new corporate and brand identity[1], marking a new milestone in its development toward the registration and commercialization of its drug candidates for rare neurological diseases.

A Major Milestone in the Company's Growth Marked by a Name Change

Since its creation, Theranexus has been committed to discovering and developing innovative solutions for patients with rare neurological diseases. By becoming **THX Pharma**, the company signals its evolution from a biotechnology company to a pharmaceutical company focused on regulatory approvals and market access for its lead drug candidates across multiple geographies. At the heart of this transformation are **TX01**, a new formulation of an already approved drug redesigned for pediatric populations with two rare diseases, and **Batten-1**, the most advanced drug candidate worldwide for the treatment of Batten disease.

"*THX Pharma embodies the evolution of our company: from laboratory innovation to bringing treatments to market for the benefit of patients. Our new identity reflects our ambition: moving up the value chain by rapidly commercializing innovative solutions in three rare diseases with high unmet medical needs. The commercial development of TX01 will be carried out both by Exeltis[2] and THX Pharma. Batten-1 will follow a streamlined, less capital-intensive development plan, thanks to the extremely encouraging efficacy data collected[3] so far. Our goal is to reach profitability by 2028. We continue to explore financing and partnership options to support the execution of our programs and accelerate our international commercial development*," said **Mathieu Charvériat, Chief Executive Officer of THX Pharma**.

[1] The company plans to submit to the next general meeting of its shareholders a change of its corporate name to THX Pharma.
[2] https://www.theranexus.com/images/pdf/Theranexus_PR_Partnership_Exeltis_VDEF.pdf
[3] https://www.theranexus.com/images/pdf/Theranexus_PR_Strong_Postive_Real_World_data_VDEF.pdf

A Global Development of Our Portfolio

THX Pharma's roadmap is now structured around three strategic pillars:

1. TX01 Candidate

TX01 is the first formulation suitable for children of an already approved drug, currently only available in solid form. The company is preparing its commercialization in the following regions:

- **Europe, the UK, Latin America, and selected Middle Eastern countries**: THX Pharma is relying on its partnership with Exeltis, which will soon initiate the registration process for TX01 in Gaucher disease and Niemann-Pick type C disease, with the joint objective of launching commercialization in 2027.
- **United States, Canada, and Australia**: THX Pharma will directly handle the registration of TX01 for Gaucher disease and Niemann-Pick type C disease by 2027, with plans to secure distribution agreements after marketing authorization.

With this commercialization strategy, THX Pharma targets €50 million in annual peak revenues by 2031–2032.

2. Batten-1 Candidate

Batten-1 is the most advanced drug in development for the juvenile form of Batten disease, a neurodegenerative condition affecting 2,000 children worldwide and with no available treatment to date. THX Pharma recently published real-world efficacy data on the same primary endpoint required by regulatory agencies FDA and EMA. These unprecedented data on visual function enable the company to implement an optimized clinical development plan through to registration and commercialization in 2028.

- **In the United States, the company will deploy an Early Access Program (EAP)** for around 50 patients. This program will be largely self-funded through patient insurance, in line with recent FDA guidance[4].
- **In Europe**, in parallel, **the company will launch a Phase 3 open-label clinical trial** on about 20 patients over a 12-month treatment period.

Both programs will generate efficacy data to support the regulatory submissions to FDA and EMA. Additional registrations in Australia and Canada are also envisioned, targeting a broad population of Batten disease patients. The company estimates €4 million in dedicated expenses for these steps between 2026 and 2028, with market launch expected in 2028. In this indication, following a commercialization strategy similar to TX01, THX Pharma aims to generate €200 million in additional revenues by 2032–2033.

[4] https://www.fda.gov/news-events/public-health-focus/expanded-access

3. An Innovative Antisense Oligonucleotide Platform

THX Pharma's scientific team is developing an antisense oligonucleotide platform tailored to rare diseases, focusing initially on one candidate targeting autophagy activation in lysosomal diseases, and a second program for glioblastoma, a market estimated at more than €3 billion. This platform benefits from decisive support from Bpifrance as part of the PickASO project[5].

About THX Pharma

THX Pharma (Theranexus) is a pharmaceutical company specializing in treatments for rare neurological diseases. Its first drug, TX01, will soon be commercialized—particularly in Europe by Exeltis, but also in the United States, Canada, and Australia—for Niemann-Pick type C disease and Gaucher disease. Its second drug, Batten-1, targets the juvenile form of Batten disease and could become the first approved therapy for this condition. THX Pharma also has an innovative antisense oligonucleotide platform, co-developed with leading research laboratories, dedicated to rare neurological diseases.
THX Pharma, a trade name of Theranexus, is listed on Euronext Growth Paris (FR0013286259 – ALTHX).

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