THERANEXUS JOINS UP WITH SYNERLAB TO PREPARE THE COMMERCIALIZATION OF ITS PEDIATRIC FORMULATION IN NIEMANN-PICK TYPE C DISEASE

- **Theranexus has developed the first oral solution of miglustat tailored to the needs of patients with Niemann-Pick type C disease,**
- **Theranexus is preparing to register this formulation with the EMA and aims to commercialize it by 2026,**
- **Synerlab Development will be responsible for the industrial production of commercial batches,**
- **Theranexus is in advanced discussions with different industry players for the distribution and commercialization of the drug.**

Lyon, France – 17 October 2024 – 6pm CEST – Theranexus, a biopharmaceutical company innovating in the treatment of rare neurological diseases, and Synerlab Group, a contract development and manufacturing organization (CDMO), have announced the signing of a partnership agreement under which Synerlab Development will manufacture the first commercial batches of the exclusive pediatric formulation of miglustat, developed by Theranexus. EMA approval will firstly be sought for this proprietary formulation for Niemann-Pick type C disease with commercialization expected to begin by 2026.

Theranexus has patented and validated the very first oral liquid formulation of miglustat in connection with its development in Batten disease. The new project based on this exclusive innovative dosage form will enhance Theranexus' portfolio and also runs in parallel with the Batten project, thereby offering a new pathway for leveraging Theranexus' work to date. This oral solution, dubbed Mig-OS, offers significant benefit for treating patients with Niemann-Pick type C[1] (NPC) disease as it offers a new therapeutic option for pediatric and/or dysphagic patients.

Miglustat is the only treatment approved in Europe for NPC, a severe neurodevelopmental disease affecting almost 1,500 patients in Europe, including 71% of patients with a pediatric form. The only formulation available to date is a 100 mg capsule, which is poorly suited to treating this young patient population, 70% of whom have severe swallowing difficulties (dysphagia).

With its expertise in both formulation and industrial production, Synerlab Developpement will be in charge of production and validation of commercial batches of Mig-OS, pursuant to the applicable EMA regulations. During the second half of 2025, Theranexus will then proceed with submitting a centralized authorization application to the EMA aiming for a commercial launch by the end of 2026. Theranexus may opt for a type of authorization known as PUMA (pediatric-use marketing authorization), which grant 10 years of commercial exclusivity providing similar protection to that of drugs with orphan designation.

For Theranexus Chairman and CEO, Mathieu Charvériat: "*Our formulation provides an innovation for patients with Niemann-Pick type C disease, and we are pleased with our partnership with Synerlab Development, a company that has demonstrated great agility and expertise in the industrial production of our drug. The funding needs for this project are substantially lower than those needed for our Batten disease project, less than €2 million for approval in Europe, with earlier potential financial and significant returns for the company. The addressable market in NPC is estimated at €200 m, with a strong exclusivity provided by our industrial property and PUMA status in Europe. We are exploring various financing avenues and are already in very advanced discussions with several industrial partners to commercialize the product in different regions, starting with Europe. Beyond its high financial interest in a relatively short term for the company, this project will also offer an opportunity to continue development in Batten disease*".

[1] Niemann-Pick type C is a rare lysosomal disease that disrupts lipid metabolism, causing severe progressive neurological disorders, such as coordination or cognitive deficiencies.

"We are proud to collaborate with Theranexus for the production of the first commercial batches of this new miglustat formulation. This partnership reflects our commitment to support the development of innovative treatments that address critical medical needs, in particular for rare diseases. With our expertise and GMP standard-compliant facilities, we are making every effort to guarantee high-quality production and help deliver this new therapeutic option to patients suffering from these lysosomal diseases", **concluded Alexandra Lecourbe, general manager of Synerlab Development.**

About Theranexus

Theranexus is an innovative biopharmaceutical company that emerged from the French Alternative Energies and Atomic Energy Commission (CEA). 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).

About Synerlab

Synerlab is a European contract manufacturing and development organization, offering a full range of services, from formulation to production. With its recognized expertise in the manufacture of drugs for specific medical conditions, Synerlab pledges to support its partners in the development of safe and efficient treatments that comply with the most stringent quality standards.

In light of the acquisition transaction underway with Blue Wolf Capital Partners, subject to regulatory clearances, the new European platform, comprising Synerlab and seven sites acquired from Recipharm, is ramping up its capacity in the production of clinical batches and micro series with two dedicated sites in Sweden in addition to Synerlab Développement in Orléans.

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