



THX Pharma and the Beyond Batten Disease Foundation Announce the Grant of a European Patent Protecting the Use of Batten-1 in Juvenile Batten Disease (CLN3)

- *A patent jointly owned by BBDF, Baylor College of Medicine and Cardiff University, and exclusively licensed worldwide to THX Pharma*
- *Protection in Europe through July 2041, complementing THX Pharma's proprietary formulation patent and the orphan drug designations granted in Europe and the United States*

Lyon, France – Austin, Texas, USA – September 8, 2026 – 6:00 p.m. CEST – THX Pharma (Theranexus), an innovative biopharmaceutical company developing treatments for rare neurological diseases, and the Beyond Batten Disease Foundation (BBDF) today announced that the European Patent Office (EPO) has granted European patent No. EP 4 188 371, entitled "Miglustat alone or in combination with trehalose for the treatment of a lysosomal storage disorder." The patent protects the use of miglustat, the active ingredient in Batten-1, for the treatment of juvenile Batten disease (CLN3).

The patent is jointly owned by the Beyond Batten Disease Foundation, Baylor College of Medicine (Houston, Texas) and Cardiff University (United Kingdom). It protects research funded by BBDF and conducted in the laboratories of Dr. Marco Sardiello (Baylor College of Medicine) and Dr. Emyr Lloyd-Evans (Cardiff University). In cellular and animal models of CLN3 disease, this research demonstrated Batten-1's ability to reduce lysosomal accumulation of glycosphingolipids, neuroinflammation and neuronal death.

Under the agreement signed by the two partners in late 2019, BBDF, which holds the exclusive rights to exploit this patent, granted THX Pharma an exclusive worldwide license for the development and commercial exploitation of Batten-1. The patent designates 38 contracting states to the European Patent Convention, including all European Union Member States, the United Kingdom, Switzerland and Norway, and provides protection through July 30, 2041.

Batten-1 Exclusivity Protection Based on Three Complementary Pillars

The grant of this patent forms part of the strategy implemented by THX Pharma and its partners to secure long-term exclusivity for Batten-1 through three independent and complementary pillars:

1. **A therapeutic-use patent.** The newly granted European patent protects the use of miglustat in the treatment of juvenile Batten disease, regardless of the dosage form used.
2. **A proprietary formulation patent.** Batten-1 is also protected by the patent portfolio entitled "Palatable liquid solution containing high concentration of miglustat," which is wholly owned by THX Pharma. Developed by THX Pharma's teams, this high-concentration, palatable oral solution of miglustat is specifically designed for pediatric administration.
3. **Orphan drug designations in Europe and the United States.** Miglustat was granted orphan drug designation for juvenile Batten disease by the U.S. Food and Drug Administration (FDA) in August 2020 and by the European Commission, following a favorable opinion from the European Medicines Agency (EMA), in October 2020.



The FDA also granted the program Rare Pediatric Disease Designation. Subject to marketing authorization, these designations provide for ten years of market exclusivity in the European Union and seven years in the United States, independently of patent protection.

Together, these three layers of protection are designed to secure Batten-1's position beyond 2040. They represent a valuable component of the global licensing agreement signed with Biocodex in February 2026 for the development and commercialization of Batten-1, and come as regulatory applications for the Batten-1-02 Phase 3 clinical trial have just been submitted to the FDA, the MHRA and, for the European Union, through the Clinical Trials Information System (CTIS).

Mathieu Charvériat, Chairman and Chief Executive Officer of THX Pharma, said: *"The grant of this European patent marks an important milestone in strengthening Batten-1's intellectual property position. It complements the protection afforded by our proprietary liquid formulation and the orphan drug designations granted on both sides of the Atlantic. Following the recent submission of regulatory applications for our pivotal Phase 3 trial, this enhanced protection reinforces the value of the program for our partners and, ultimately, our ability to ensure sustainable access to treatment for children affected by this disease."*

Craig Benson, Founder and Chairman of the Board of the Beyond Batten Disease Foundation, commented: *"This patent is the result of more than ten years of research funded by our foundation alongside teams at Baylor College of Medicine and Cardiff University. It illustrates the strategy BBDF has pursued from the outset: investing in science, securing its results and entrusting them to an industry partner capable of turning them into a treatment. Its grant in Europe protects this long-term investment and supports the continued development of this innovation for the benefit of families affected by Batten disease."*

About THX Pharma (Theranexus)

THX Pharma (Theranexus, Euronext Growth: ALTHX) is a biopharmaceutical company specializing in the development of innovative treatments for rare neurological diseases. The company is developing in particular TX01, a new formulation intended for the treatment of Gaucher disease and Niemann-Pick disease type C, and Batten-1, a drug candidate targeting juvenile Batten disease (CLN3). TX01 is the subject of several licensing agreements with international pharmaceutical partners, including Exeltis and Biocodex, for its development and commercialization in different regions of the world. Batten-1 is covered by a global licensing agreement with Biocodex for its development and commercial exploitation. THX Pharma also has an innovative antisense oligonucleotide platform, co-developed with leading research laboratories, dedicated to the development of new therapeutic approaches for rare neurological diseases. THX Pharma is listed on the Euronext Growth market in Paris (ISIN: FR0013286259 – ALTHX).

For more information:

<http://www.thxpharma.com>

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About the Beyond Batten Disease Foundation (BBDF)

The Beyond Batten Disease Foundation (BBDF) is a nonprofit organization founded in Austin, Texas, in 2008 by Craig and Charlotte Benson following the diagnosis of their daughter Christiane with juvenile Batten



disease (CLN3). Under the leadership of its Founder and Chairman of the Board, Craig Benson, BBDF is dedicated to eradicating juvenile Batten disease by raising public awareness and securing the funding needed to accelerate the development of treatments and the search for a cure. The Foundation also brings together families, researchers and industry partners around a coordinated scientific strategy.

For more information, visit: www.beyondbatten.org.

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Forward-Looking Statements

This press release contains forward-looking statements relating to THX Pharma (Theranexus), the Beyond Batten Disease Foundation (BBDF) and their respective activities, including the scope, validity, national validation, maintenance and enforceability of European patent EP 4 188 371 and other intellectual property rights relating to Batten-1; the effective duration of Batten-1 exclusivity; the granting and maintenance of regulatory exclusivities associated with orphan drug designations; and the timing, conduct and results of the Batten-1 Phase 3 clinical trial and the prospects of the program. THX Pharma and BBDF believe that these forward-looking statements are based on reasonable assumptions. However, forward-looking statements are not guarantees of future performance. They are based on future events and are subject to circumstances that may or may not occur, as well as to various risks and uncertainties, including those described in the Universal Registration Document filed by the Company with the French Financial Markets Authority (Autorité des marchés financiers – AMF) on April 29, 2026, under number D.26-0321, available on the Company's website, as well as changes in economic conditions. The grant of a patent does not guarantee its validity if challenged or the absence of third-party rights. Batten-1 is an investigational drug candidate and has not been approved by any regulatory authority. Neither THX Pharma nor BBDF undertakes any obligation to update or revise any forward-looking statements contained in this press release, except as required by applicable laws or regulations.