



THX Pharma, BBDF and Biocodex Announce Regulatory Submissions for the Phase 3 Clinical Trial of Batten-1 in Juvenile Batten Disease (CLN3)

Lyon, France – Austin, Texas, USA – Gentilly, France – September 1, 2026 – 6:00 p.m. CEST – THX Pharma (Theranexus), an innovative biopharmaceutical company developing treatments for rare neurological diseases, together with its partners, the Beyond Batten Disease Foundation (BBDF) and Biocodex, today announced the submission of regulatory applications to initiate the Phase 3 clinical trial evaluating Batten-1 (study Batten-1-02), a proprietary oral liquid formulation of miglustat developed for the treatment of juvenile Batten disease (CLN3).

The regulatory applications have been submitted to the U.S. Food and Drug Administration (FDA), the UK Medicines and Healthcare products Regulatory Agency (MHRA), and, for the European Union, through the Clinical Trials Information System (CTIS), covering the competent authorities of the participating European countries (France, Germany, the Netherlands, Denmark, Sweden and Italy). Subject to regulatory approvals, patient enrollment is expected to begin by year end.

Batten-1-02: An International Pivotal Phase 3 Trial

Batten-1-02 is an open-label, single-arm, multicenter Phase 3 clinical trial designed to demonstrate the efficacy of miglustat, administered as the proprietary oral liquid formulation Batten-1, in pediatric patients with juvenile Batten disease (CLN3). The study's primary endpoint is the assessment of visual acuity, a standardized, validated endpoint recommended by the FDA.

Approximately 27 patients aged 4 to 17 years with the syndromic form of CLN3 disease who retain measurable visual acuity in at least one eye at baseline will receive treatment for 52 weeks. Visual acuity outcomes in patients treated with Batten-1 will be compared with data from natural history studies conducted over several years by the National Institutes of Health (NIH) in the United States and the University Medical Center Hamburg-Eppendorf (UKE) in Germany.

Based on previous clinical results, the expected therapeutic benefit is the stabilization of visual acuity after one year of treatment in patients receiving miglustat, compared with untreated patients. The study is fully funded by Biocodex under the global licensing agreement for Batten-1 announced in February 2026. It will be conducted in 10 specialized Batten disease reference centers across the United States and Europe.

Mathieu Charvériat, Chairman and Chief Executive Officer of THX Pharma, said: *"The submission of these regulatory applications marks a major milestone for the Batten-1 program and for families affected by Batten disease, for which no approved treatment is currently available. This achievement reflects the commitment of all our partners—including expert clinical centers and patient advocacy organizations—working toward a shared objective: demonstrating, through this international pivotal trial, Batten-1's ability to slow the decline in visual acuity in children affected by this rare neurodegenerative disease. We would like to express our sincere gratitude to the families, investigators, and patient organizations whose commitment has made this program possible."*



Craig Benson, Founder and Chairman of the Board of the Beyond Batten Disease Foundation, commented: *“Every family participating in this study does so with the hope that science will arrive in time for their child. The submission of these regulatory applications brings us one step closer to that moment—when the first patients can be enrolled in the trial. As the world’s largest patient organization dedicated to juvenile Batten disease, we take very seriously the responsibility entrusted to us by families since our foundation in 2008. We recognize both how far we still have to go and how much progress has already been made through the dedication of our partners and the clinical teams working across multiple continents.”*

Nicolas Coudurier, Chief Executive Officer of Biocodex, added: *“We are reaching an important milestone in the implementation of our global licensing agreement with THX Pharma. Under this agreement, Biocodex provides scientific and financial support for the Batten-1 program, while the Phase 3 study is being led by THX Pharma in collaboration with expert clinical centers across North America and Europe, alongside the Beyond Batten Disease Foundation. This commitment builds on more than thirty years of expertise in rare neurological diseases and prepares the next stage of our mission: supporting future market access and making this treatment available to children living with juvenile Batten disease and their families.”*

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.

About Biocodex

Biocodex is an independent French pharmaceutical company operating in more than 100 countries through its subsidiaries and partners. A pioneer in the exploration of the gut microbiota, the Group has made this field a central strategic focus, driven by the conviction that this ecosystem is key to a more holistic approach to health.

The Group develops innovative healthcare solutions across four complementary areas: microbiota, women's health, rare diseases, and established products addressing common healthcare needs, including mental health, sleep disorders and pain management. True to its family-owned heritage, Biocodex is committed to advancing healthcare by combining scientific excellence, a strong industrial footprint in France, international reach and corporate social responsibility. This commitment has been recognized through the Engagé RSE label (Confirmed Level – ★★), awarded by AFNOR Certification and recognized across Europe through Responsibility Europe. **“Empowering everyone to live fully”** is Biocodex's purpose, embraced by its 1,800 employees worldwide.

For more information, visit: <https://www.biocodex.com>



Contacts:

THX PHARMA

Christine PLACET

Chief Financial Officer

contact@thxpharma.com

THX PHARMA / FP2COM Agency

Florence PORTEJOIE

Media Relations

+ 33 (0)6 07 76 82 83

fportejoie@fp2com.fr

BIOCODEX / Monet Agency

Anne-Lise Le Vaillant/Florence Desremaux

+33 (0) 1 45 63 12 43

rpbiodex@monet-rp.com

BEYOND BATTEN DISEASE FOUNDATION

Mary Beth Kiser

mbkiser@beyondbatten.org



Forward-Looking Statements

This press release contains forward-looking statements relating to THX Pharma (Theranexus), the Beyond Batten Disease Foundation (BBDF), Biocodex and their respective activities, including the expected initiation, timing, design and results of the Phase 3 clinical trial of Batten-1, future regulatory submissions and the prospects of the Batten-1 program.

THX Pharma, Biocodex 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.

Batten-1 is an investigational drug candidate and has not been approved by any regulatory authority. Neither THX Pharma, Biocodex 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.