



JOINT PRESS RELEASE

THX Pharma, Biocodex and the Beyond Batten Disease Foundation Stand Together with the CLN3 Batten Community on International Batten Disease Awareness Day

The three partners are proud to jointly serve as Platinum Sponsors of the 2026 BDSRA Annual Family Conference, where they will provide an update on the Batten-1 program and the upcoming initiation of their international pivotal Phase 3 study

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– On the occasion of International Batten Disease Awareness Day happening today, THX Pharma (Theranexus), a biopharmaceutical company specializing in the development of innovative treatments for rare neurological diseases, Biocodex, an independent international pharmaceutical group and long-time pioneer in the treatment of rare pediatric diseases, and the Beyond Batten Disease Foundation (BBDF), a patient-founded non-profit dedicated to eradicating juvenile Batten disease, today reaffirm their shared commitment to the patients, families, caregivers and clinicians affected by CLN3 Batten disease.

Each June 9, the global Batten community comes together to raise awareness of a group of rare, inherited neurodegenerative disorders that primarily affect children and young adults — and for which therapeutic options remain extremely limited. In juvenile (CLN3) Batten disease, families typically witness the onset of vision loss in the first decade of life, followed by seizures, progressive motor decline, cognitive deterioration and premature death. There is, to date, no approved disease-modifying treatment for CLN3 Batten disease.

Joint Platinum Sponsorship of the 2026 BDSRA Annual Family Conference

To mark this year's awareness day, THX Pharma, Biocodex and BBDF are proud to announce that they will jointly serve as Platinum Sponsors of the 2026 BDSRA Annual Family Conference, hosted by the Batten Disease Support, Research and Advocacy Foundation (BDSRA Foundation) from July 10 to 12, 2026, at The Westin Chicago Lombard (Illinois, USA). The BDSRA Annual Family Conference is the largest gathering of Batten disease families, clinicians, researchers and industry partners in North America and an essential forum for community connection, education and the exchange of the most recent scientific advances.

Representatives from BBDF, THX Pharma and Biocodex will be present throughout the conference. They will engage directly with families and members of the broader Batten community and will jointly deliver an update on the Batten-1 program, a miglustat-based investigational therapy designed to slow the progression of CLN3 Batten disease — including the planned initiation, later this year, of the international pivotal Phase 3 clinical study intended to support future regulatory submissions.



“International Batten Disease Awareness Day is, for all of us at THX Pharma, a moment of unity with the families and patients and motivation for us. The progress made from the positive Phase 1/2 results, through the data confirming a clinical benefit on visual function, to the imminent launch of our phase 3 study — has only been possible thanks to the trust and commitment of the CLN3 community and of our partners BBDF and Biocodex. We are honored to stand alongside them in Chicago this July,” said **Mathieu Charvériat, President and Chief Executive Officer of THX Pharma.**

“By joining BBDF and THX Pharma as a Platinum Sponsor of the BDSRA Annual Family Conference, Biocodex is reaffirming a principle that has guided its commitment to rare diseases for more than three decades: meaningful progress in rare pediatric neurology is built together with patient communities. We are deeply committed to advancing Batten-1 with the ambition of making this treatment available to patients by 2029, with the scientific rigor that the families affected by CLN3 disease deserve” said **Nicolas Coudurier, Chief Executive Officer of Biocodex.**

“For our families, every June 9 carries both grief and resolve. Eighteen years after BBDF was founded, we are now on the verge of something we could only dream of in 2008: an international pivotal Phase 3 trial for a candidate therapy aimed at slowing CLN3 Batten disease. To see THX Pharma and Biocodex join us as Platinum Sponsors of the 2026 BDSRA Conference is a powerful signal to our community that we are not walking this road alone,” said **Craig Benson, Founder and Chairman of the Beyond Batten Disease Foundation.**

About Batten-1 and the upcoming Phase 3 study

Batten-1 is an investigational, orally administered, miglustat-based therapy being developed for the treatment of CLN3 (juvenile) Batten disease. The 18-month results of the Phase 1/2 Batten-1-01 study (NCT05174039), conducted in six young adult patients with CLN3 Batten disease, demonstrated a favorable safety and pharmacokinetic profile, alongside encouraging biomarker and clinical signals — including a slowdown of motor symptom progression as measured by the modified UBDRS Physical Assessment score and a reduction in neurofilament light chain (NfL) levels, a recognized biomarker of neurodegeneration. These findings were further reinforced in 2025 by real-world data, generated in collaboration with BBDF, suggesting a clinical benefit of miglustat on visual acuity in CLN3 patients.

Building on this body of evidence, the partners are now preparing the initiation, in 2026, of the international pivotal Phase 3 clinical study of Batten-1. The study, funded by Biocodex under the terms of the global licensing agreement announced in February 2026, is intended to support future Marketing Authorization Applications in major regions in 2029, with the objective of bringing Batten-1 to patients as quickly and as broadly as possible.



About the Beyond Batten Disease Foundation (BBDF)

Beyond Batten Disease Foundation (BBDF) is the world's largest nonprofit organization dedicated to funding research for a treatment and cure for juvenile (CLN3) Batten disease. Since its inception in 2008, over \$35 million has been invested in research by leveraging donations, co-funding and strategic partnerships. BBDF is spearheading a unique, cohesive strategy, incorporating independent scientific resources and collaboration with related organizations to drive research in juvenile Batten Disease. Today there is a treatment in sight. BBDF funded research has discovered a drug – Batten-1 – that slows the progression of the disease in Batten models. More information can be found at www.beyondbatten.org.

About Biocodex

Biocodex is an independent French pharmaceutical group founded in 1953, present in 115 countries through its subsidiaries and partners. A long-time pioneer in the treatment of rare pediatric diseases — most notably with Diacomit® (stiripentol) in Dravet syndrome — Biocodex has built a recognized expertise in pediatric neurology and orphan diseases. Through long-term partnerships with biotechnology innovators and patient communities, the Group is committed to developing and bringing to patients effective treatments that address high unmet medical needs. More information at biocodex.com.

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 (CLN3) Batten disease. 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).). <https://www.theranexus.com>



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Forward-Looking Statements

This press release contains forward-looking statements relating to THX Pharma (Theranexus), Biocodex, the Beyond Batten Disease Foundation, and their respective activities, including the planned initiation, timing, design and results of the international pivotal Phase 3 clinical study 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 as of the date of this press release. However, no assurance can be given that the expectations expressed in such forward-looking statements will be achieved. These statements are subject to risks and uncertainties, including those related to clinical development, regulatory review, manufacturing, commercialization and the financing of investigational therapies. Actual results may differ materially from those anticipated. 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.