



THXpharma

Rare focus, real impact.

MARCH 17th, 2026 – CF&B Small Cap Event

THX Pharma is a commercial brand from THERANEXUS 

Our D.N.A

We serve rare disease patients with innovative treatments



3 patents for 2 solutions :
TX01 & **Batten-1**



2 other solutions in development



2 partnerships with global pharmas

* THX Pharma is a commercial brand of Theranexus (ALTHX) to enact our transition from a biotech to a pharma-model

Roadmap

2024

Licence agreement with **Exeltis** to commercialize **TX01** in Europe, LATAM & MENA

2026

Licence agreement with **Biocodex** to develop and commercialize **TX01** in US & Canada and **Batten-1** worldwide.

2027

First sales for **TX01** by both Exeltis and Biocodex

2029

First sales for **Batten-1** by Biocodex

Our pipeline in a nutshell

Commercialisation in 2027



TX01

Niemann-Pick
Type C
& Gaucher
Disease



Europe, LATAM, MENA



NorthAm

Launching Phase III & early access

Program fully funded by Biocodex

Commercialisation end 2029

Batten-1

Batten disease



Worldwide rights

Two assets at non-clinical stage

**R&D
platform**







Institut national
de la santé et de la recherche médicale

bpifrance



TX01: taking on 2 Inherited disorders in children

Niemann–Pick C (NPC) and Gaucher Disease (GD)

	Niemann–Pick C	Type 1 Gaucher
	Mutation in NPC1 or NPC2 genes	Mutation in GBA1 gene
	<ul style="list-style-type: none"> • Difficulties in eye movement • Difficulties in coordination and motor skills including swallowing • Cognitive / language impairments 	
	20,000 patients in major markets	
 current therapies	<ul style="list-style-type: none"> • Enzyme replacement therapies (ERT) • Substrate reduction therapies (SRT) 	Treatment with Zavesca® (miglustat) in solid form Supplemented with <ul style="list-style-type: none"> • Miplyffa® (arimoclomol) • Aqneursa® (levacetylleucine)

TX01 is based on an approved drug for both diseases and developed in solid form by other pharmas

TX01 provides a convenient product for both populations:

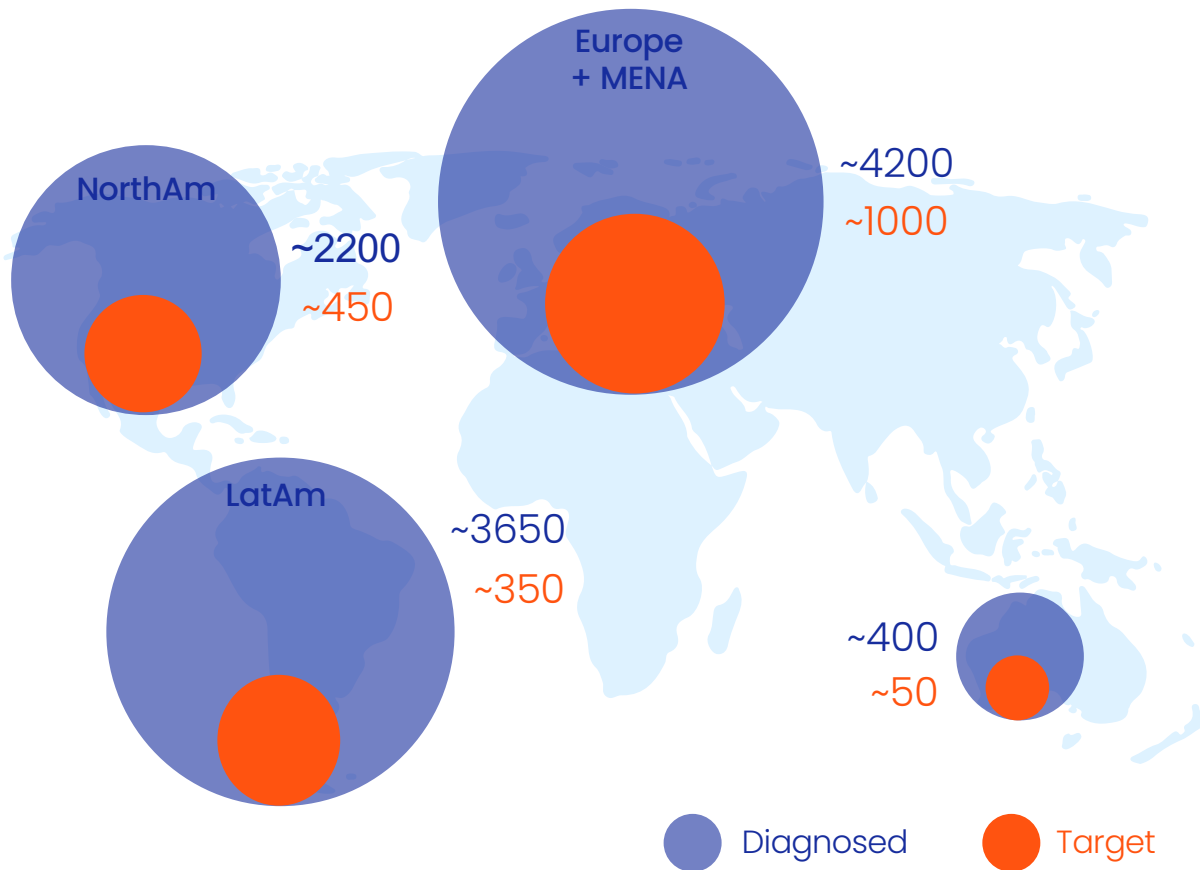


A unique & **proprietary formulation** of an already approved drug



Technical package already **available for market approval** applications

TX01: Towards commercialisation in 2027



TX01 has been exclusively licensed to :

- Biocodex (US, Canada) in 2026
- Exeltis (Europe, LATAM, MENA) in 2024

In other territories, THX will file for approvals (2026) and commercialize TX01 through distribution agreements (2027).

First sales in 2017, forecasted +50m€ turnover by 2032



In the US, drug prices for the target indications range from \$200K to \$1.2M per patient per year.

Batten-1: Tackling a rare fatal disorder with no approved therapy

Juvenile Batten disease (CLN3)

Pathophysiology:

Batten Disease is a mutation in CLN3 gene, leading to the buildup of toxic glycosphingolipids (GSL) in the brain/eye, leading to neuronal cell death.

Development & Symptoms:



4-6 years old

Vision decline leading to blindness



6-8 years old

Cognitive decline leading to dementia



10-12 years old

- Epileptic seizures
- Speed & motor decline



20s

Patients pass away



**No treatment to date,
no drugs approved.**

Batten-1 is the most advanced drug in the development pipeline for Batten disease. Our closest active competitor is in early phase 1.

Batten-1: The first drug to tackle juvenile Batten disease

An oral solution adapted to CLN3 patient needs



Batten's API (miglustat) is **already approved** in other indications in all major markets



Suitable for both pediatric and dysphagic patients, with dynamic dosage to suit all ages and needs



Targets toxic glycosphingolipid accumulation, **significantly slowing most symptoms**

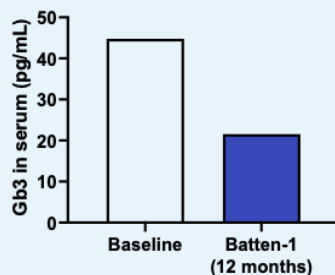


Batten-1 can be the **first drug approved** in the indication

Batten-1: Clinical findings

1

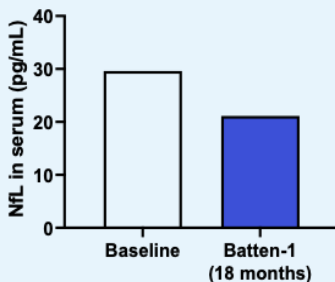
Batten-1 targets and reduces GSL in CLN3 patients*



Batten-1 blocks GSL accumulation (-50%) in patients, also demonstrated in NPC and GD patients

2

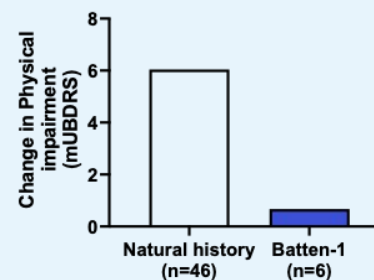
Batten-1 reduces neuronal cell death in CLN3 patients*



By blocking GSL accumulation, **Batten-1 blocks neuronal cell death in patients by -33%**

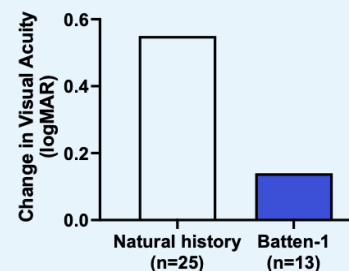
3

Stabilization of motor function*



Phase 1/2
NCT05174039
n=6 CLN3 patients,
aged 17+

Stabilization of visual acuity (RWE)

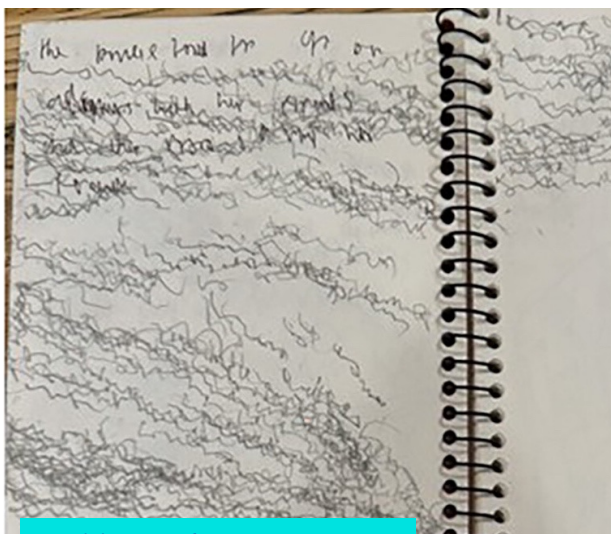


Stabilisation of visual acuity loss compared to natural history (ANCOVA, $p=0.07$)

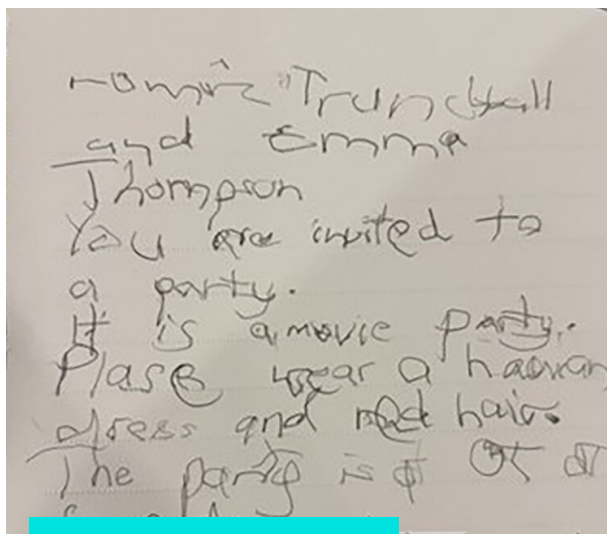
Reduction by -60% the risk of visual acuity deterioration (log reg, $p=0.08$)

* Reference: NCT05174039

Batten-1: What does it mean for patients?



Writing before treatment



Same patient,
Writing after 12 months

Case study:

11 years old girl in Australia suffering from CLN3 Batten*

After treatment with miglustat :

- Improvement in visual behavior
- Identifying friends, color, objects
- Interactions with complex games
- More legible handwriting
- Improvement of school performance

* Dutton et al, *Journal of Child Neurology*, Sept 2025

Batten-1: Building strong value with Biocodex partnership

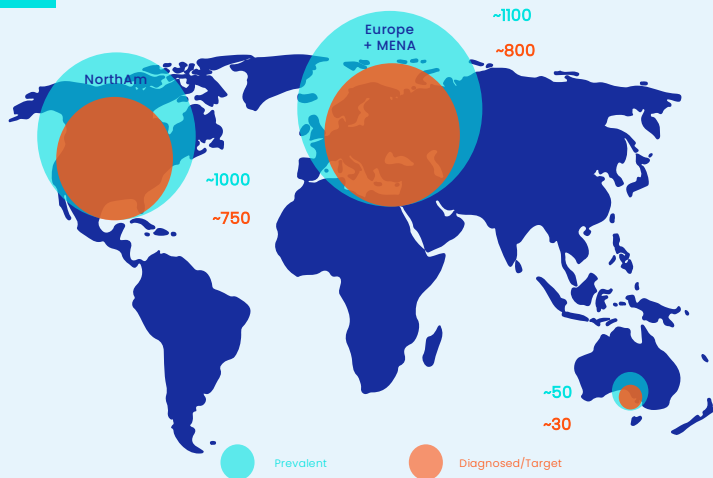
1



In the US, drug prices in comparable indications range from \$400K to \$750K per patient per year.

2

A targeted population of +1500 patients



A global 500 €m annual market

4

Batten-1 is licensed to Biocodex :



- A world-wide exclusive licence to a pharma established in +100 countries
- +170m€ potential milestone, and two-digit royalties on sales
- A total ROI more favorable than a direct commercialisation by THX Pharma

3



A phase 3 and an EAP launched in 2026 and financed by Biocodex



A primary endpoint already endorsed by the FDA and the EMA



Commercialization planned for 2029

Our R&D platform

dedicated to antisense oligonucleotides (ASO) in rare diseases

We work with **various partners** to identify & characterize targets in rare diseases and new ASOs:



Our platform enables us to **develop new projects** for the future:

1

THX-ASO1: Our first non-clinical candidate

- The first ASO targeting TFEB pathway, promoting autophagy. A development funded by **Bpifrance** (4.7 m€), in collaboration with **Inserm and Diverchim**.
- Clinical development planned for 2028, **with potential across 10+ indications**

2

THX-ASO2: Targeting an undisclosed pathway in glioblastomas

Glioblastomas, a 3 bn€ market in 2024

Financial projections & funds management

Resources fully dedicated to the development of our assets

Proforma cash position: 22,7 m€
(December 31st, 2025)

A current cash runway extending **beyond 2028**



In €K (French GAAP)	H1 2025	H1 2024
Operating income	817	2,221
Other purchases and external charges	1,043	1,294
Wages and social security charges	855	1,073
Amortization and depreciation	48	47
Other operating expenses	22	32
Operating expenses	1,968	2,446
Net operating income/(expenses)	-1,151	- 226
Net financial income/(expense)	1	36
Income tax	39	0
Net income/(expense)	41	- 291
	- 1 072	101

First half 2025 income statement

Key take-aways

THX Pharma is strongly committed to both patients & shareholders



Our motivation : transforming the lives of patients with rare diseases



Innovative new formulations adapted to children and patients experiencing difficulties to swallow



New therapies in rare genetic disorders



Clinical programs aiming at fast commercialisation of our candidates



Our commitment to our shareholders :



2026–2029: dense newsflow with multiple value catalysts.



A company no longer requiring additional fund raising



Building strong value on two strategic partnerships

Contact us:

Mathieu Charvériat, CEO
mathieu.charveriat@thxpharma.com

Christine Placet, CFO
christine.placet@thxpharma.com

thxpharma.com



[THX Pharma](https://www.linkedin.com/company/thx-pharma)



EURONEXT GROWTH INFORMATION:

ISIN: FR0013286259
Ticker symbol: ALTHX

