
THXpharma
Rare focus, real impact.
SEPT 30, 2025 – EUROPEAN MIDCAP EVENT 2025
THX Pharma is a commercial brand from THERANEXUS

Our D.N.A

Building on Theranexus' medical achievements, THX Pharma* serves rare disease patients with innovative treatments, while creating shareholder value through a strategy designed to optimize ROI within 3 years.

3 patents for 2 solutions : TX01 & Batten-1

2 other solutions in development

1 commercial partnership with a global pharma

Roadmap:

2027 1st commercial sales

2028 Break even

2031 € 50 M turnover

2032 € 250 M turnover

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

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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
	• Difficulties in eye movement • Difficulties in coordination and motor skills including swallowing • Cognitive / language impairments	
	20,000 patients in major markets	
current therapies	• Enzyme replacement therapies (ERT) • Substrate reduction therapies (SRT)	Treatment with Zavesca® (miglustat) in solid form Supplemented with • Miplyffa® (arimoclomol) • Aqneursa® (levacetylleucine)

A single agent has been approved for both diseases and developped in solid form by other pharmas,

with 2 major drawbacks:

- no formulation is suitable for dysphagic and/or pediatric patients
- no formulation is offered in a format that allows for dynamic dosages

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TX01: Solution

An new formulation designed to be more convenient to GD and NPC patients

A new and **proprietary formulation** of an already approved drug

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

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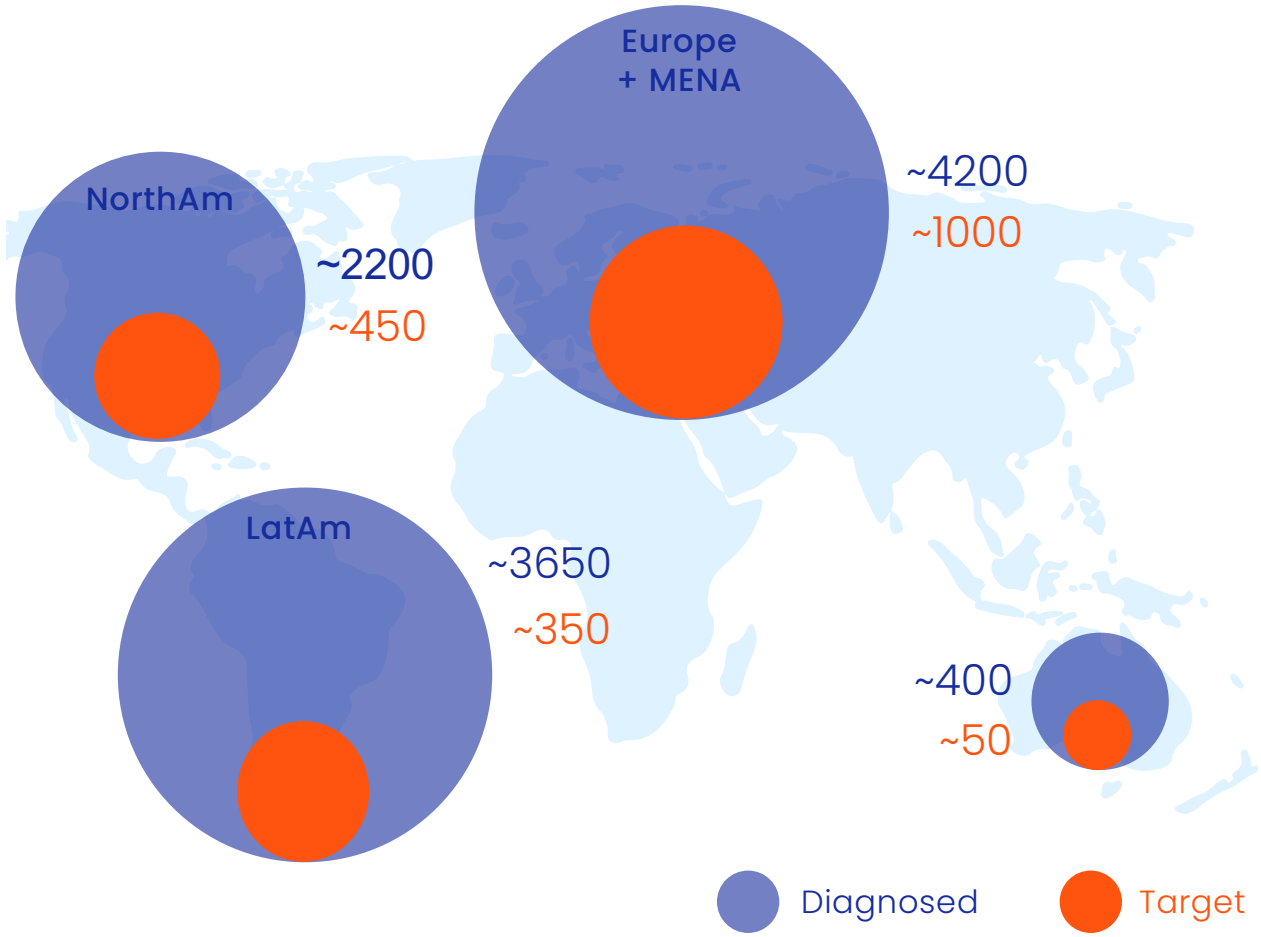
TX01:
Towards 50 Mn€ in revenue by 2032



Europe, LATAM, MENA: Our product has been licensed to Exeltis, a pharma company ($2Bn revenue) with global sales in 50+ countries, **through an exclusive license** (2024), with significant development milestones and royalties

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

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

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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.

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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

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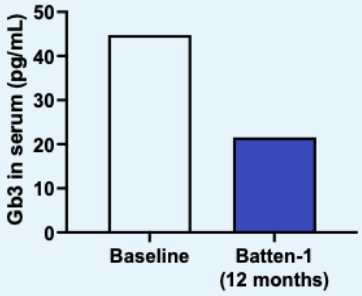
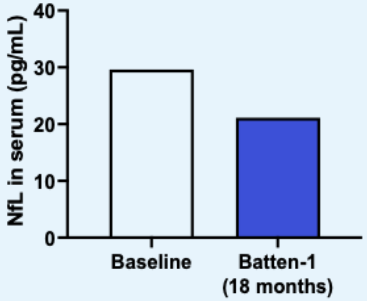
Batten-1: Clinical findings

1 Batten-1 targets and reduces GSL in CLN3 patients*



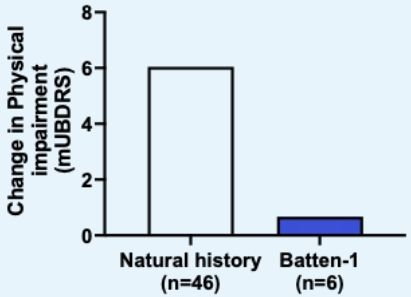
Batten-1 blocks GSL accumulation(-50%) in patients, also demonstrated in NPC and GD patients

2 Batten-1 reduces neuronal cell death in CLN3 patients*



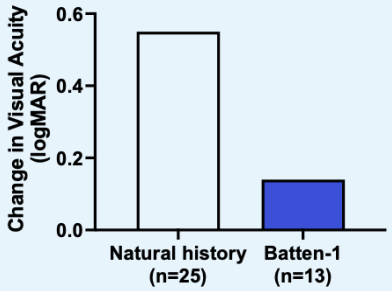
By blocking GSL accumulation, **Batten-1 blocks neuronal cell death in patients by -33%**

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

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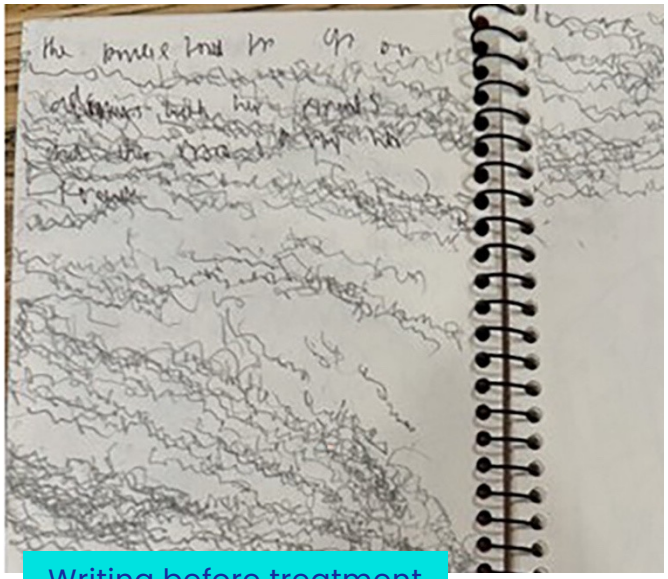
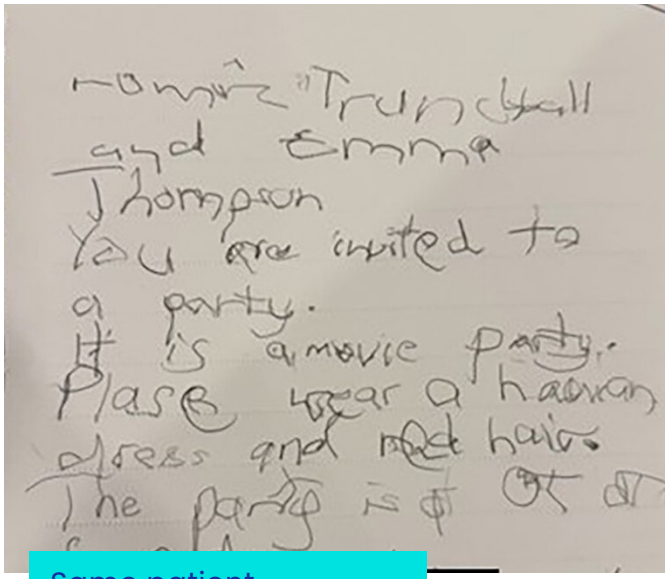
Batten-1: What does it mean for patients?



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

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

Batten-1: An optimized road toward market approval in 2028

Early Access Program

Launch Q1 2026

n=50 CLN3 patients with measurable visual fonction left at treatment initiation.

Funding will largely come from insurance reimbursement for patient care.

Phase 3 single-arm open label study (with external comparison)

Launch Q1 2026

n=20 CLN3 patients with measurable visual fonction left at baseline. 12 months of treatment.

An optimized clinical program

- Partnership with the US-based Beyond Batten Disease Foundation, the world's largest advocacy group for CLN3 Batten disease patients
- Primary endpoint already endorsed by the FDA and the EMA
- Commercialization planned for 2028, with moderate capital needs (~4 m€) compared to previous strategy (~10 m€)
- Avoiding the burden of the placebo arm previously considered
- A pathway frequently applied in rare diseases – 15+ known precedents.

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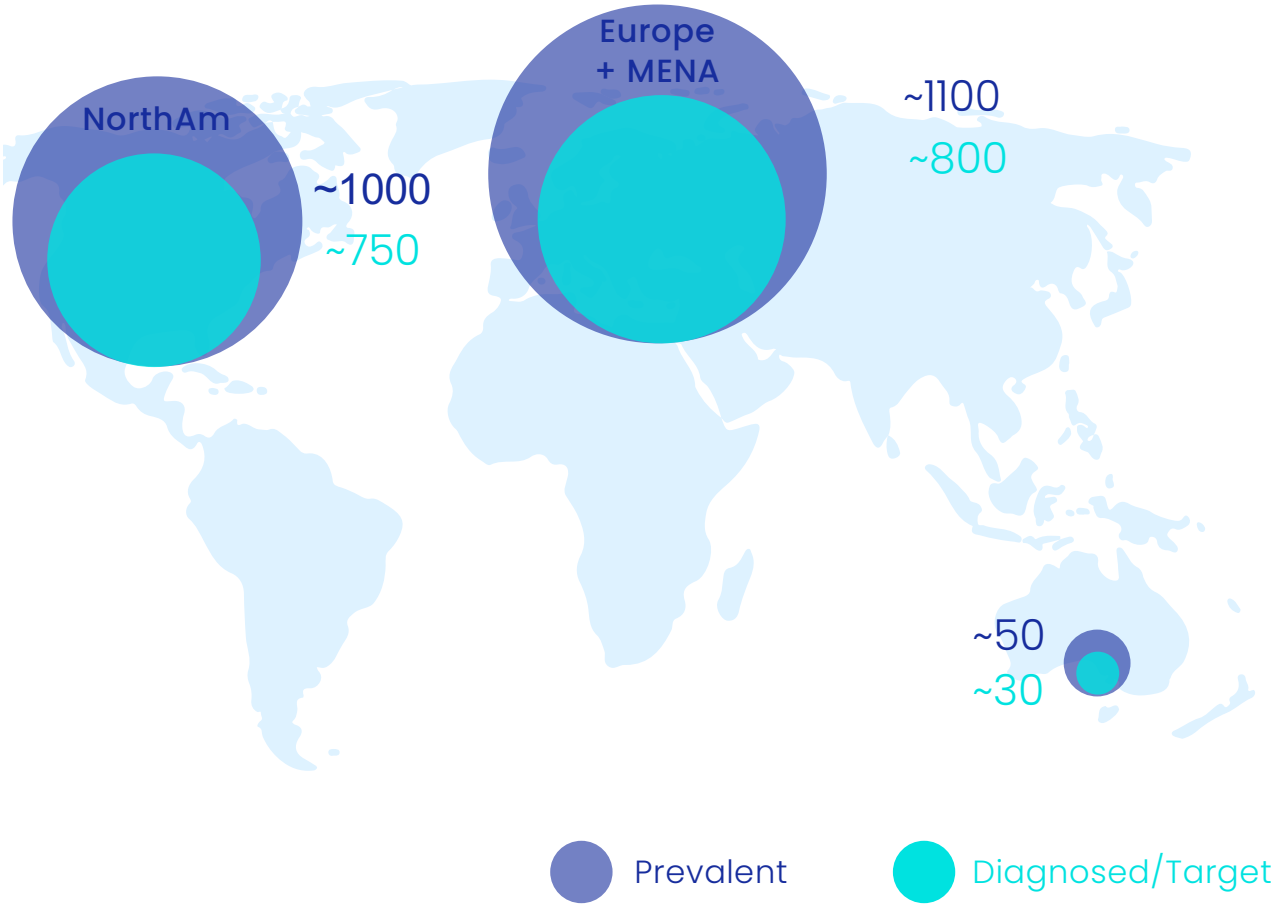
Batten-1:
Towards 200 Mn€ in revenue by 2033



Orphan drug designations (EMA, FDA) together with two families of patents, grant exclusivity up to 2047.

THX pharma plans to file for approvals in all major markets for Batten disease in 2028 and join forces with partners for **commercialization soon after in late 2028.**

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

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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:

Target discovery & validation | **ASO validation**

AI for new target and ASO identification **(with Inria)**

Identification of **innovative ASO** in rare neurological diseases and target validation **(with Inserm)**

Pathological models of rare neurological diseases for ASO characterization **(with CEA)**

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 indisclosed pathway in glioblastomas

Glioblastomas, a 3 bn€ market in 2024

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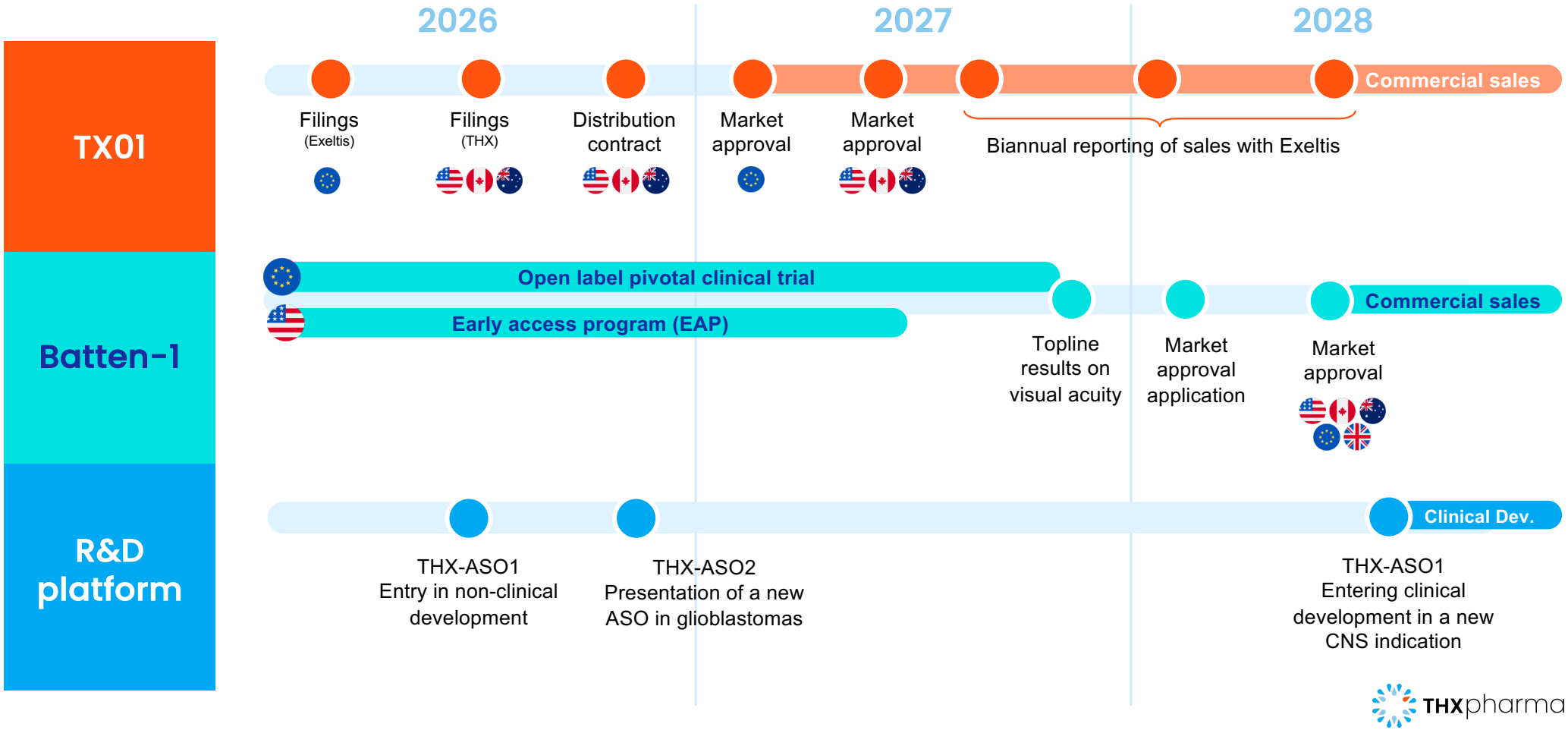
2026-2028, a pivotal period for Theranexus

Towards our break-even point



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Who is THX Pharma?

1 A biotech that morphed into a pharma

Since our beginnings, we have developed innovative solutions designed to improve the lives of underserved young patients.
Today, this commitment drives us to take the next step: becoming a pharmaceutical company.

2 A company rooted in top-tier research

We are part of ongoing programs backed by leading research institutions and hospitals:

Inserm · cea · Inria · NIH National Institutes of Health · Baylor College of Medicine · Texas Children's Hospital · Cardiff University · Prifysgol Caerdydd · Beyond Batten Disease Foundation

3 A team of dedicated specialists, led by experts

Mathieu Charvériat
CEO & Chairman

- 20+y in neuroscience and drug dev.
- Ecole Polytechnique, PhD, Mines Paris Tech

Julien VEYS
COO

- 20+y in drug dev.
- MBA HEC Paris
- Former CBO of Trophos (sold to Roche - 470 M€)

Christine PLACET
CFO

- 30+y in drug dev.
- Former CEO of Trophos & Horama (now Coave)

4 A well-placed, publicly listed company

We are listed on the Euronext Growth market (**ALTHX**) :

- +294% *vs* Jan 1st, 2025 (as of Sept 27th, 2025)
- Ranked 1st in performance in the Boursorama Health category (since Jan 1st, 2025)
- Analyst: BNPP Portzamparc

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Financial projections & funds management

Our resources are fully dedicated to the development of our assets

Cash position: 2,1 m€ (June 30, 2025)

Our current cash runway extends **beyond Q2 2026**

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

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Key take-aways

THX Pharma is strongly commited 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 :

- 2026–2028: dense newsflow with multiple value catalysts.
- Break-even expected in 2028 — a rare achievement in our field.
- Forecasting 250 Mn€ in annual revenue by 2032, with continued growth thereafter.

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