THERANEXUS

INVESTOR PRESENTATION

Sachs Life Sciences Forum
March 8 & 9, 2023

Disclaimer

This document contains certain forward-looking statements concerning Theranexus and its business, including its prospects and product candidate development. Such forward-looking statements are based on assumptions that Theranexus considers to be reasonable. However, there can be no assurance that the estimates contained in such forward-looking statements will be verified, which estimates are subject to numerous risks including the risks set forth in the universal registration document of Theranexus filed with the AMF on 29 April 2022 under number D.22-0399 D.21-0379 (a copy of which is available on www.theranexus.com) and to the development of economic conditions, financial markets and the markets in which Theranexus operates. The forward-looking statements contained in this press release are also subject to risks not yet known to Theranexus or not currently considered material by Theranexus. The occurrence of all or part of such risks could cause actual results, financial conditions, performance or achievements of Theranexus to be materially different from such forward-looking statements. Theranexus expressly declines any obligation to update such forward-looking statements.

Speakers

Mathieu CHARVERIAT

Co-founder& CEO

Ecole Polytechnique and Mines Paris-Tech

Doctor in Neuroscience and Cell Biology from the Pierre and Marie Curie Institute.

HEC Challenge + program

Thierry LAMBERT

Chief Financial Officer

Business Administration at the University of Birmingham

MBA (Master of Business Administration) from INSEAD

Chartered Accountant and Certified Public Accountant ICAEW (Institute of Chartered Accountants in England and Wales)

Theranexus is a clinical-stage company developing drug candidates targeting rare neurological diseases

Batten-1

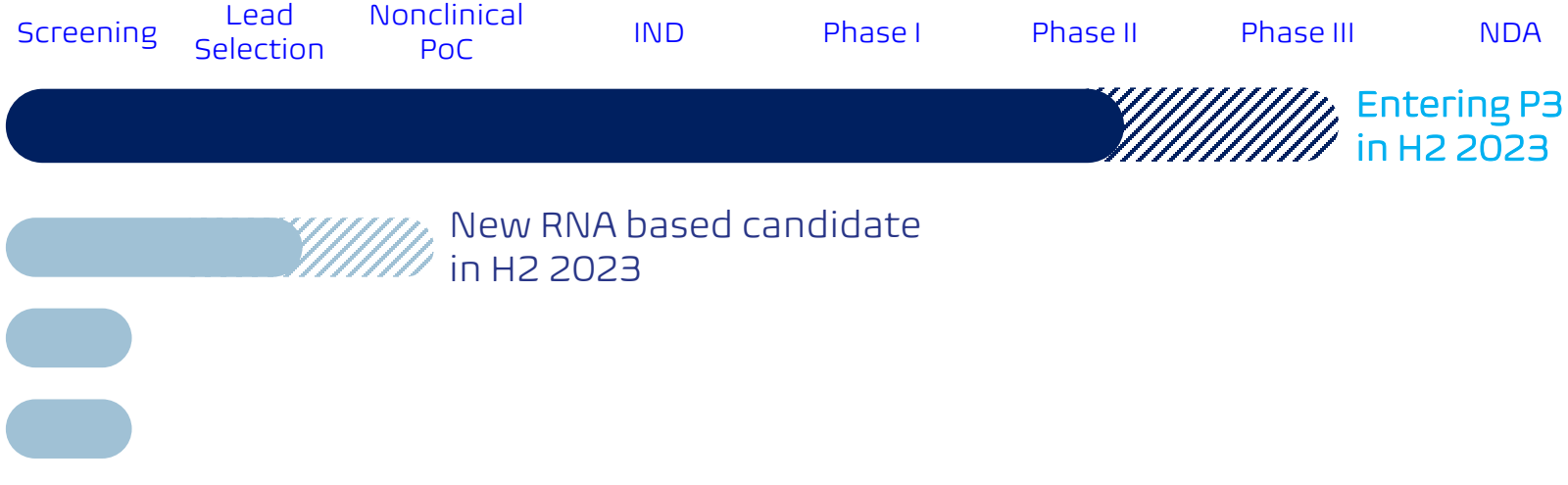
BEYOND BATTEN DISEASE FOUNDATION

Juvenile Batten disease (CLN3)
P1-2 fully recruited & ongoing

TFEB platform

Lysosomal dysfunction platform

Neuronal hyperexcitability platform



The R&D platforms is notably built upon 3 scientific collaborations :

Neurolead

cea

COLLÈGE DE FRANCE 1530

Drug candidates identification platform based on pathological models of rare neurological diseases

INSERM

Inserm
La science pour la santé
From science to health

Identification of innovative drug therapy candidates (RNA)
In rare neurological disorders

AlstroSight

Inria INVENTEURS DU MONDE NUMÉRIQUE

HCL HOSPICES CIVILS DE LYON

Université de Lyon | Université Claude Bernard Lyon 1

Digital simulation and AI platform to assist the identification of therapeutical targets to treat rare neurological disorders

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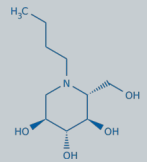
A first highly promising asset in Juvenile Batten disease (CLN3)

Recent understanding of the mechanism of the disease

- Central role of the accumulation of GSL[1] in neuronal death[2 3]

Use of an approved compound with a clinically validated mechanism of action

- Validated efficacy through the inhibition of the synthesis of GSL in patients suffering from CNS lysosomal storage disorders



A proprietary liquid formulation adapted to patients' need

- Fine-tuning of dosage
- Patents filed
- 2021 and 2022
- ODDs[4] granted by FDA & l'EMA

A crucial partnership

- BBDF : the main patient advocacy group in this field

BEYOND BATTEN DISEASE FOUNDATION

The only advanced clinical stage asset in CLN3

- Safety profile already validated in CLN3 patients (P1/2)
- Launch of a US/Europe P3 in H2 2023

First-to-market in a market with strong potential

- Approval in 2026
- >500 M$ peak sales

[1]Glycosphingolipids
[2]Work performed by the laboratory of Emyr Llyod Evans at Cardiff University (financed by Beyond Batten Disease foundation)
[3] Lloyd-Evans E, et al. Glycosphingolipid reduction with miglustat as a therapeutic strategy for CLN3 and other neuronal ceroid lipofuscinoses WORLD symposium 2023
[4]Oprhan Drug Designation

CLN3 : the mechanism of the disease was discovered thanks to recent academic work funded by BBDF

700 - 1 000 patients in the US[1]
800 - 1 000 patients in the EU[2]

Autosomal recessive
Founder effect located in the Nordic countries

No treatment approved

CLN3 protein mutation → Buildup of toxic glycosphingolipids in the brain → **Neuronal cell death** leads to highly disabling neurological symptoms and death of the patients

- Loss of visual acuity leading to to blindness 6-10 years
- Cognitive decline and epileptic seizures 12-20 years
- Motor symptoms before the age of 20 Patients pass in their 20s

Scientific rationale leading to the discovery of Batten-1 :
How to reduce the buildup of these toxic glycosphingolipids ?

[1] Based on US insurance claims (Decision Resources Group)
[2]: Based on Orphanet data
[3] Work performed by the laboratory of Emyr Llyod Evans at Cardiff University (financed by Beyond Batten Disease Foundation)

Batten-1 targets the synthesis of glycosphingolipids which accumulation is toxic to neurons

Miglustat (active ingredient of Batten-1) blocks the synthesis of GSL, leading to a reduction of their accumulation

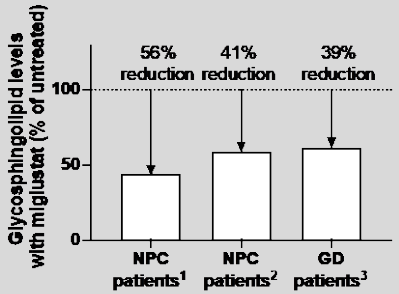
This pharmacology is validated in the clinic in other indications

Reducing neuronal cell death, with the objective of stopping the progression of the disease

GSL levels greatly reduced (clinical demonstration in similar indications)

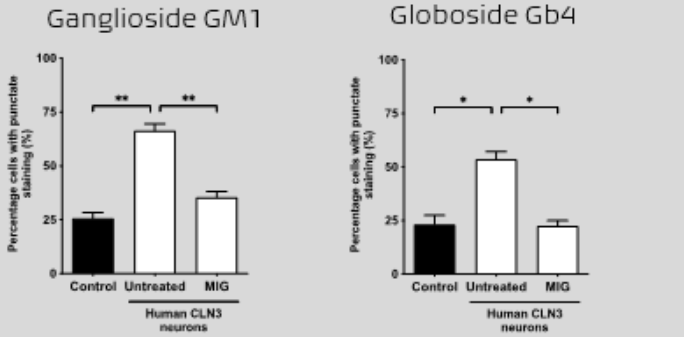
Miglustat (Active ingredient of Batten-1) already proved its clinical efficacy in reducing the level of GSL in two indications:

- Gaucher disease
- Niemann-Pick type C disease



[1] Lachmann et al, 2004
[2] Fan et al, 2013
[3] Zavesca labeling package, EMA, 2005

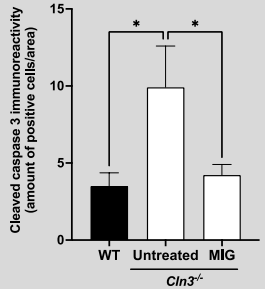
Accumulation of GSL greatly reduced in CLN3



CLN3 cellular models[1]

A normalized cell death in CLN3

Apoptosis



Modèles vivo CLN3[2]

Batten-1 is effective in targeting the synthesis of glycosphingolipids and normalizing cell death[1 2]

1: Work performed at Emyr Lloyd-Evans' lab (Cardiff University, UK) funded by BBDF
2: Work performed at Marco Sardiello's lab (Baylor College of Medicine, US) funded by BBDF

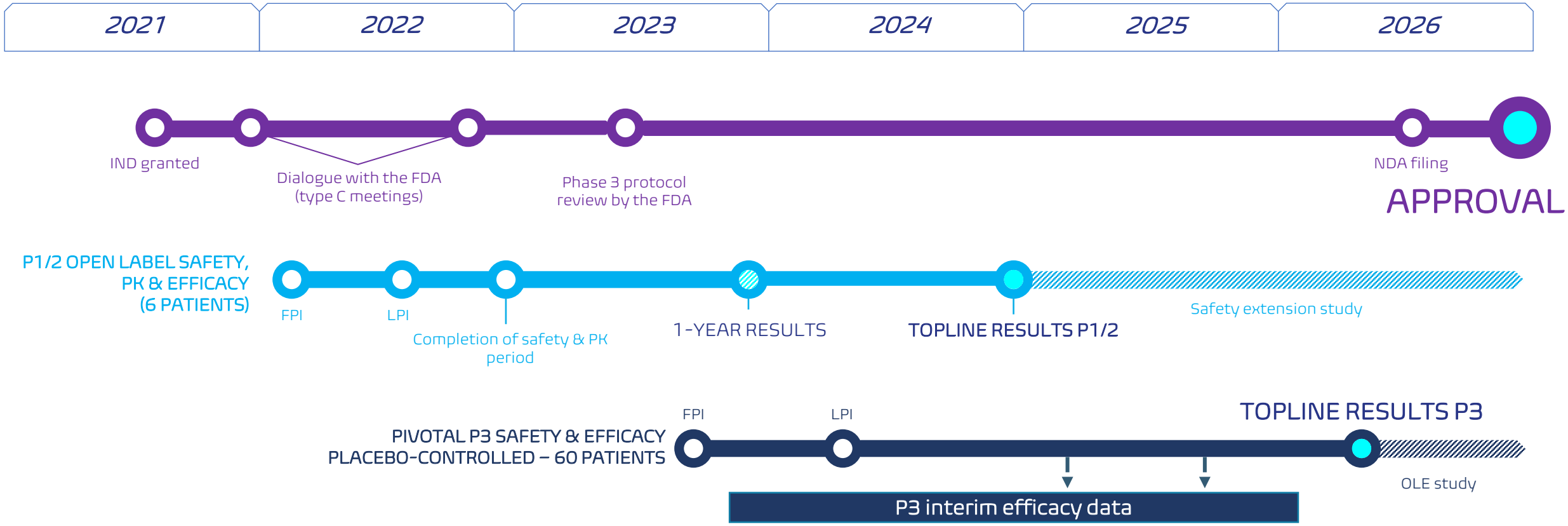
Batten-1: development of a proprietary drinkable formulation adapted to the CLN3 patient population

- **A drinkable solution that is more convenient to take and modulate the treatment**
  - Drinkable solution :
    - Suitable for pediatric patients as well as for patients presenting difficulties in swallowing
    - High concentration, allowing treatment of all patients regardless of age or weight
  - Graduated pipette => fine modulation of the dose, allowing the best possible tolerance during the progressive increase in dose at the beginning of the treatment

- **Method of use and formulation patents** filed in major markets (2021 & 2022)

- **Orphan Drug Designations granted by the FDA (US) and the EMA (EU)**

Development plan with the ambition of a market approval by 2026



Regulatory

Phase 1/2 study in CLN3 patients aged ≥ 17 y.o.

Phase 3 study in CLN3 patients aged 4 y.o to 16 y.o

AIM FOR A MARKET APPROVAL BY 2026

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Batten-1: global peak sales exceeding $500 million per year

Territories	no. of currently diagnosed patients	Sales based on diagn. patients *(USD m)*[1]	Est. prevalence[4]	Sales based on est. patients *USD m)*[1]
United States	~750[2]	~480	~1'200	~765
UE5 (Germany, France, Italy, Spain, UK)	~340[3]	~50	~500	~75
Nordic countries	~120[3]	~15	~180	~20
MARKET OPPORTUNITY		$545 Mn		$860 Mn

1: Estimated pricing based on payers and KOLs interviews conducted in Q1 2022 (source: Justin Stindt Consulting report for Theranexus)

2: Estimated diagnosed population based on insurance reimbursement claims for neuronal ceroid lipofuscinosis (NCL) (ICD-10 code: E75.4) with an age at diagnosis between 8 and 15 y.o.; (source: DRG - Clarivate analytical report for Theranexus).

3: Estimated diagnosed population based on data derived from hospital discharges reports for neuronal ceroid lipofuscinosis (NCL) (ICD-10 code: E75.4) with an age at diagnosis between 8 and 15 y.o in the UK and Germany (RW Health report for Theranexus)

4: Estimates based on analysis of the literature (DRG - Clarivate analytical report for Theranexus ; RW Health report for Theranexus)

Theranexus : a strong value creation proposition for investors

Batten-1, a particularly attractive clinical-stage asset:

- ✓ Batten-1 registration in 2026
- ✓ A well-understood mechanism with already demonstrated efficacy
- ✓ A high degree of protection (proprietary formulation + ODD)
- ✓ A facilitated access to a high-potential market (>$500M annually):
  - Partnership already in place with BBDF (the largest CLN3 patient advocacy group worldwide)
  - Streamlined process until registration
  - Limited number of prescribers and possibility of early access to accelerate treatment adoption
- ✓ No treatment on the market and no active development in the clinic

An R&D platform dedicated to rare neurological diseases:

- ✓ The platform builds upon top academic collaborations
- ✓ A new RNA candidate in H2 2023

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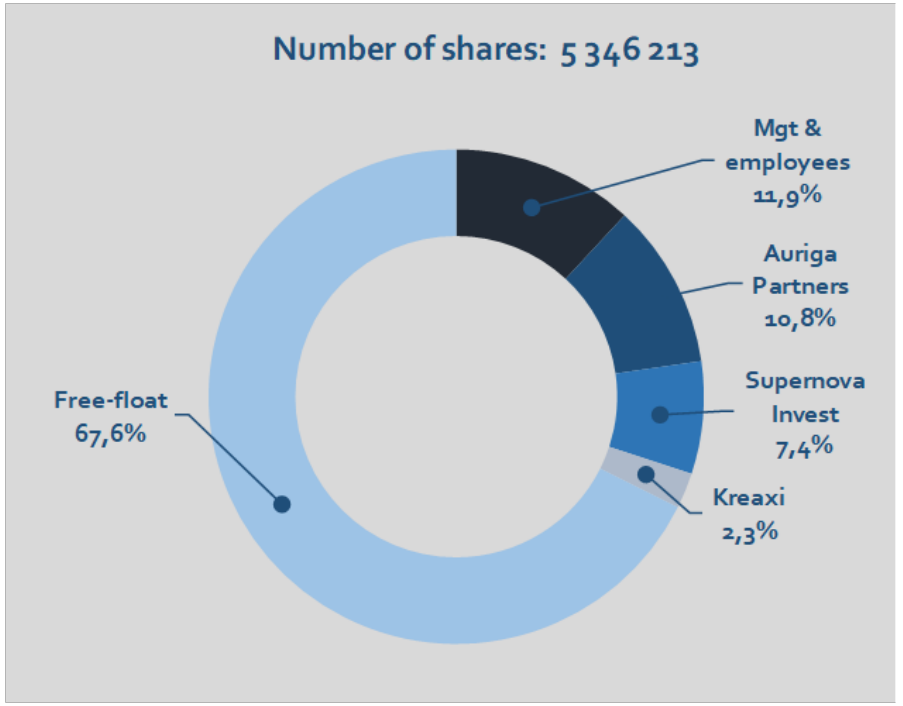
Appendices

Listing and Shareholding

Financial data

- ISIN : FR0013286259 - Ticker: ALTHX
- Market: Euronext Growth
- Share price on 22 February 2022: €1.91
- Market capitalization: €10.2m
- Coverage: ODDO BHF, Portzamparc
- Liquidity contract: Portzamparc

Capital structure

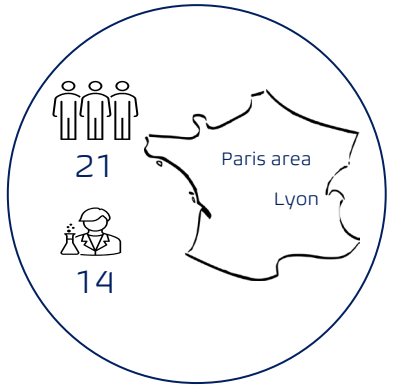


Simplified income statement

Controlled expenditure

In K€ (French standards)	S1 2022	S1 2021
Operating income	**462**	**12**
Other purchases and external charges	1 950	2 944
Wages and social charges	1 509	1 388
Depreciation and amortization on fixed assets	166	195
Other expenses	24	34
Operating result	**(3 187)**	**(4 550)**
Financial result	(121)	(112)
Tax on profits	18	939
Net result	**(3 290)**	**(3 723)**

Cash and cash equivalents at Dec. 31st 2022: €9,140k



THANKS FOR YOUR ATTENTION !

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