THERANEXUS

INVESTOR PRESENTATION

January 2023

Speakers

Julien VEYS

Chief Business Development Officer

Graduate in Science from the University of Aix-Marseille

MBA (Master of Business Administration) from HEC Paris

20 years of experience in the healthcare industry

Mathieu CHARVERIAT

Co-founder, CSO & Deputy 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)

Profile and pipeline

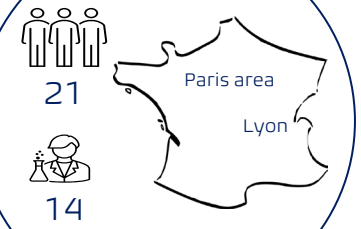
THERANEXUS IS A CLINICAL STAGE COMPANY DEVELOPING FIRST-IN-CLASS OR FIRST TO MARKET INNOVATIONS TO TACKLE RARE NEUROLOGICAL DISORDERS

2013
Spin off from an academic lab in Paris area

2017
Entered the Euronext stock market

2019
Partnership with Beyond Batten Disease Foundation

2022
Launch of P1/2 trial of Batten-1 in young adults with Batten disease



Batten-1

BEYOND BATTEN DISEASE FOUNDATION

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

TFEB platform

Lysosomal dysfunction platform

Neuronal hyperexcitability platform

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Juvenile Batten disease (CLN3) in brief

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

Autosomal recessive
Founding effect localised in the Nordic countries

No treatment registered

Diagnosis in children aged 4 to 8 years

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 die in their 20s

The absence of CLN3 leads to the **accumulation of toxic deposits of glycosphingolipids** and results in **neuronal death**

Our objective : to market the first treatment for Batten disease by 2026:

- Targeting glycosphingolipids synthesis to change the course of the disease
- with a formulation adapted to the patient population

1: based on US insurance claims (Decision Resources Group)
2: based on Orphanet data

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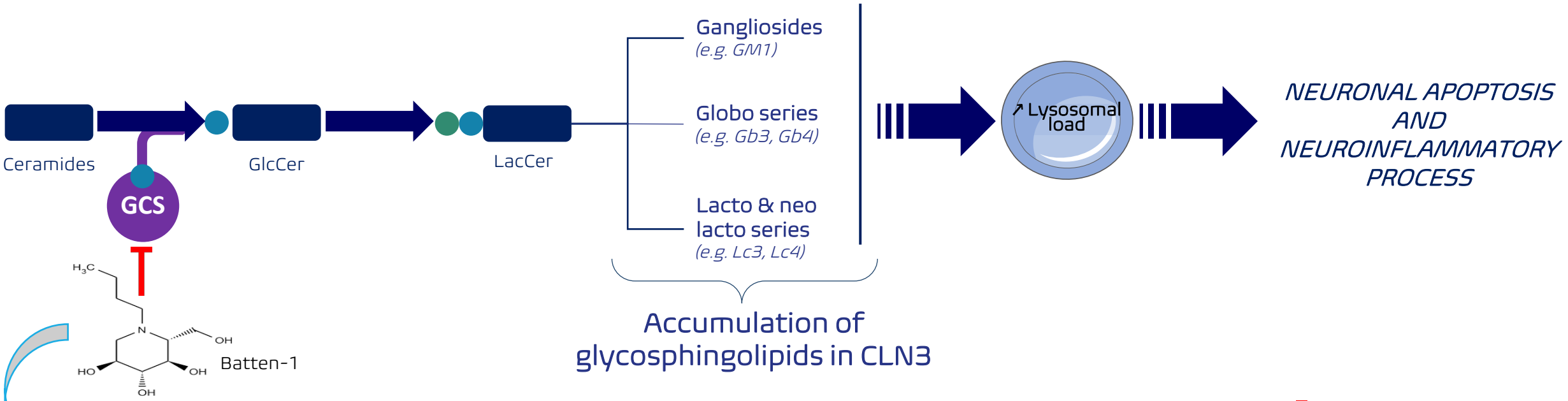
BEYOND BATTEN DISEASE FOUNDATION

Batten-1 in CLN3: a strong molecular rationale



Batten-1 (miglustat) is an inhibitor of glucosylceramide synthase (GCS), a key upstream enzyme in the synthesis of most glycosphingolipids

- Inhibition arrow
- Lactose
- Glucose

Batten-1, through its direct effect on glycosphingolipid synthesis, blocks neuroinflammation and prevents cell death in Batten disease

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BEYOND BATTEN DISEASE FOUNDATION

Batten-1 in CLN3: non-clinical validation of efficacy

Miglustat restores physiological levels of glycosphingolipids and reduces apoptosis in CLN3 models

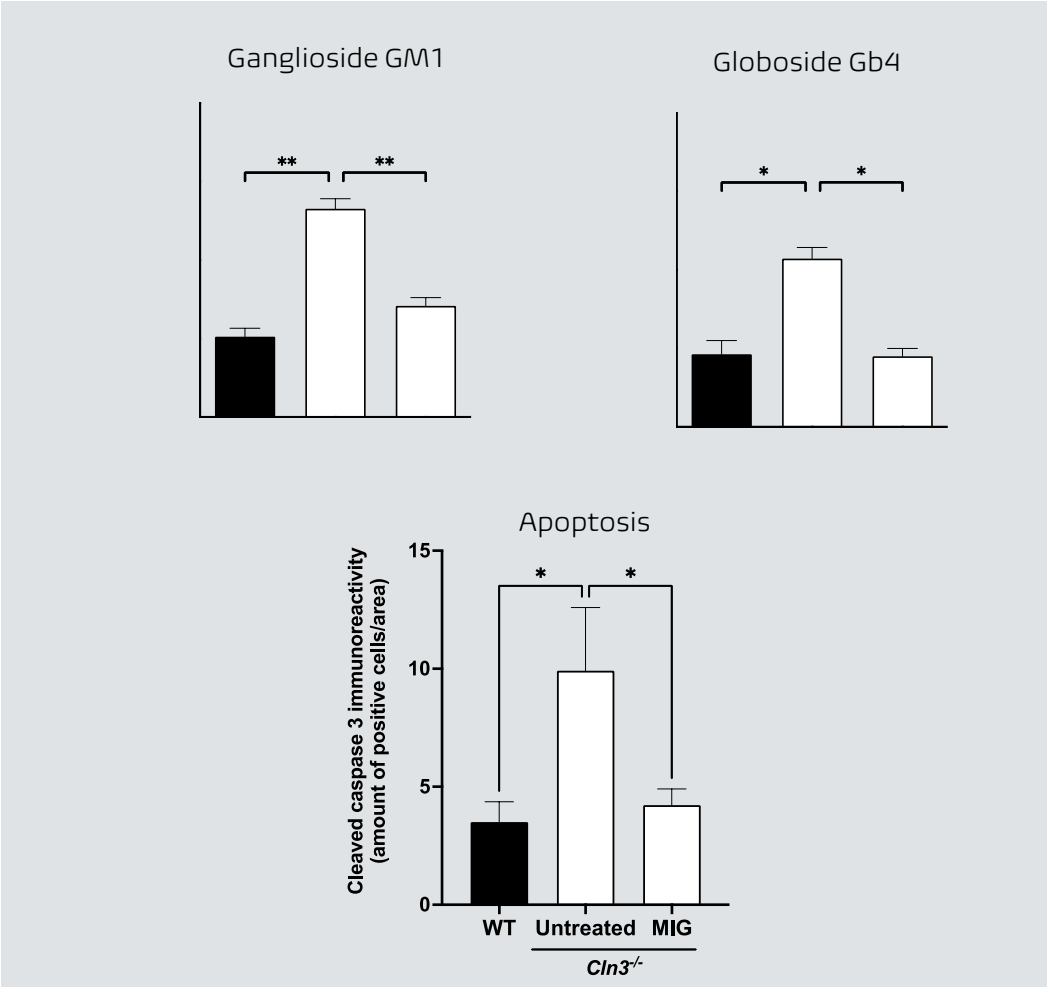
CLN3 patient iPSC derived cortical neurons :

- Reversion of GM1, Gb4, SCMAS by miglustat

Similar conclusions in several other CLN3 cellular models :

- CLN3 patient derived fibroblasts
- Neurons derived from a non-clinical model of CLN3 (mouse)
- HeLa cells with CLN3 down-regulation of expression

Miglustat reduced apoptosis (cell death) in the brain of CLN3-KO Mice



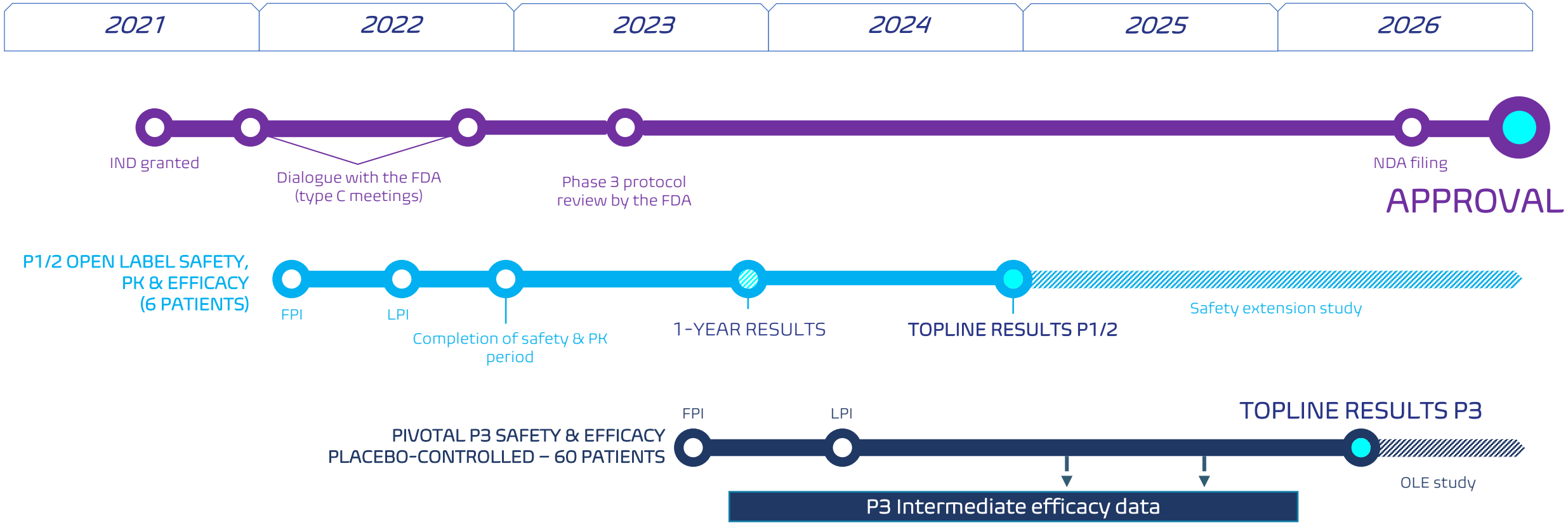
Work by Emyr Lloyd Evans' lab at the U. of Cardiff (UK) and by Marco Sardiello at Baylor College of Medecine

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

- **A drinkable solution that makes it easier 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)

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)

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Attractive competitive environment and strong market opportunities

- No competing treatments
- Two other assets in clinical development:
  - Polaryx - clinical study inactive for 2 years [1]
  - Amicus[2] , - will require further pre-clinical development

Batten-1 on the front line developments

- Orphan designation obtained from the FDA (7 y. exclusivity) and the EMA (10 y. exclusivity)
- Patent protection covering at least until 2037
- New proprietary drinkable formulation adapted to patients' needs

High level of protection

- Partnership already in place with BBDF (largest CLN3 patient advocacy group worldwide)
- Streamlined process to approval
- Limited number of prescribers
- Possibility of early access to speed up treatment adoption

Facilitated access to a 500+M$ market

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1 https://www.clinicaltrials.gov/ct2/show/NCT04637282?term=polaryx&draw=2&rank=1
2 https://clinicaltrials.gov/ct2/show/NCT03770572?cond=Batten+Disease&draw=2&rank=2

Project achievements and ambition

- ✓ ODD status for Batten-1
- ✓ IND status for Batten-1
- ✓ P1/2 launched and fully recruited
- ✓ Good tolerance of Batten-1 highlighted by P1/2 safety results
- ✓ Clinical sites selected for P3

H2. 2023	Start of the Phase 3 clinical trial with Batten-1 in children with CLN3 disease
H2. 2026	Market approval

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Appendices

Theranexus' discovery platforms

Neuronal hyperexcitability platform

Lysosomal storage disorders platform

Glial modulation of neuronal activity

Neuron/glia metabolism

Proteinopathies & autophagy

TFEB platform

Healthy & diseased iPSC derived co-cultures
neurons + astrocytes

Assessing neuron/glia interactions
in vitro & in vivo
Ca^{2+} imaging
High throughput optogenetic
3D super resolutive &
in situ imaging

in vivo validation using our imaging capabilities in small animal models
fMRI, fUS, PET

Antisense Oligonucleotides design and synthesis

Identify TARGETS OF INTEREST IN RARE CNS DISORDERS and address those with either small molecules or antisense oligonucleotides

Inserm[1]

ARNA[2]

1: French National Institute of Health and Medical Research
2: Nucleic Acids: Natural and Artificial Regulations Lab

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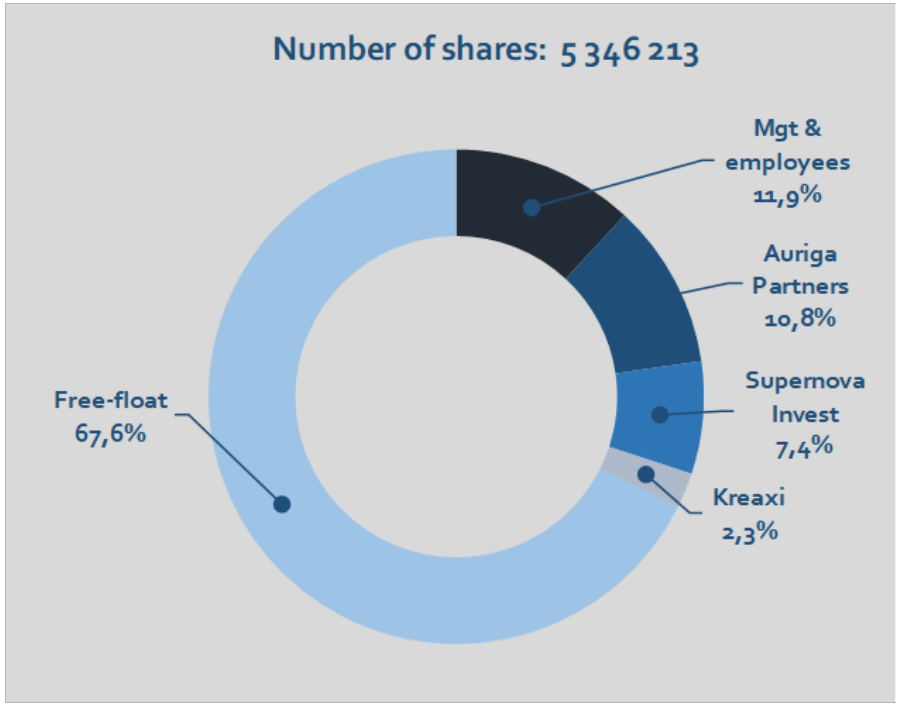
Listing and Shareholding

Financial data

- ISIN : FR0013286259 - Mnemo: ALTHX
- Market: Euronext Growth
- Share price on 30 December 2022: €1.75
- Market capitalisation: €9.3m
- 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 30/09/22: €11,000k

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THANK YOU FOR YOUR ATTENTION!

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