

# Theranexus

## INVESTORS PRESENTATION

May 2023

# Speakers



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Chief Financial Officer

Business Administration at the  
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Chartered Accountant and Certified  
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Chartered Accountants in England and  
Wales)

# Theranexus, a clinical stage company...

...developing drug candidates for rare neurological disorders

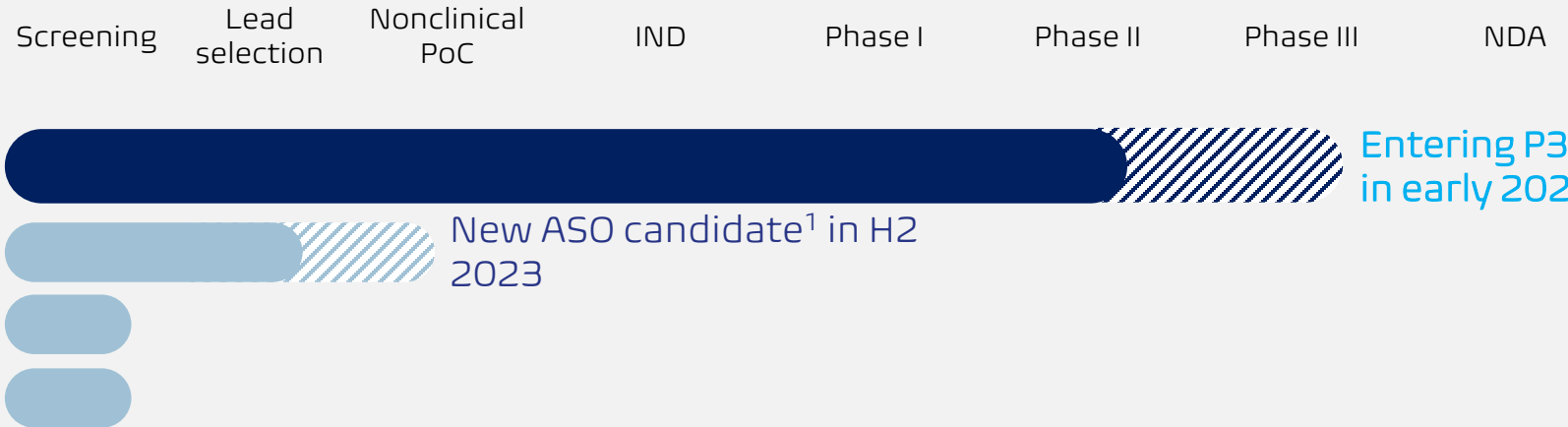


Juvenile Batten disease (CLN3)  
P1-2 fully recruited and ongoing







TFEB platform

Lysosomal diseases platform

Neuronal hyperexcitability platform



The R&D platform relies especially on  
**3 scientific collaborations:**

Neurolead	INSERM	AlstroSight
Platform for the identification of drug candidates based on pathological models of rare neurological diseases	Identification of drug candidates for innovative therapies (ASO) <sup>1</sup> in rare neurological diseases	Innovative numerical methods for the search of new drug candidates to treat rare neurological diseases
 	 <p>La science pour la santé From science to health</p>	  

# A particularly promising first active ingredient in juvenile Batten disease (CLN3)

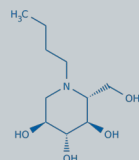
## Recent discovery of the disease mechanism

- Central role of GSL accumulation<sup>1</sup> in neuronal death<sup>2,3</sup>



## Use of a registered molecule with a clinically validated mechanism of action

- Validated ability to inhibit GSL formation in patients with lysosomal CNS diseases



## A proprietary & patient adapted liquid reformulation

- Fine adjustment of the dose
- Patents filed 2021 and 2022
- ODDs<sup>4</sup> granted by FDA & EMA



## A structuring partnership

- BBDF: the leading patient foundation in this field



## The only active agent in clinical development in CLN3

- Safety profile already validated in CLN3 patients (P1/2)
- Launch of a Phase 3 US/Europe in early 2024



## First-to-market in a high potential market

- Registration H1-2027
- >500 M peak sales



<sup>1</sup>Glycosphingolipids

<sup>2</sup>Work carried out by the laboratory of Emyr Llyod Evans at Cardiff University (funded by the Beyond Batten Disease foundation)

<sup>3</sup> Glycosphingolipid reduction with miglustat as a therapeutic strategy for CLN3 and other neuronal ceroid lipofuscinoses, Dr Emyr Lloyd-Evans, Cardiff University

<sup>4</sup>Orphan drug designation

# CLN3: the mechanism of the disease has been elucidated thanks to recent academic work funded by BBDF

## In a nutshell



700 to 1 000 patients in the US<sup>1</sup>



800 to 1 000 patients in the EU<sup>2</sup>

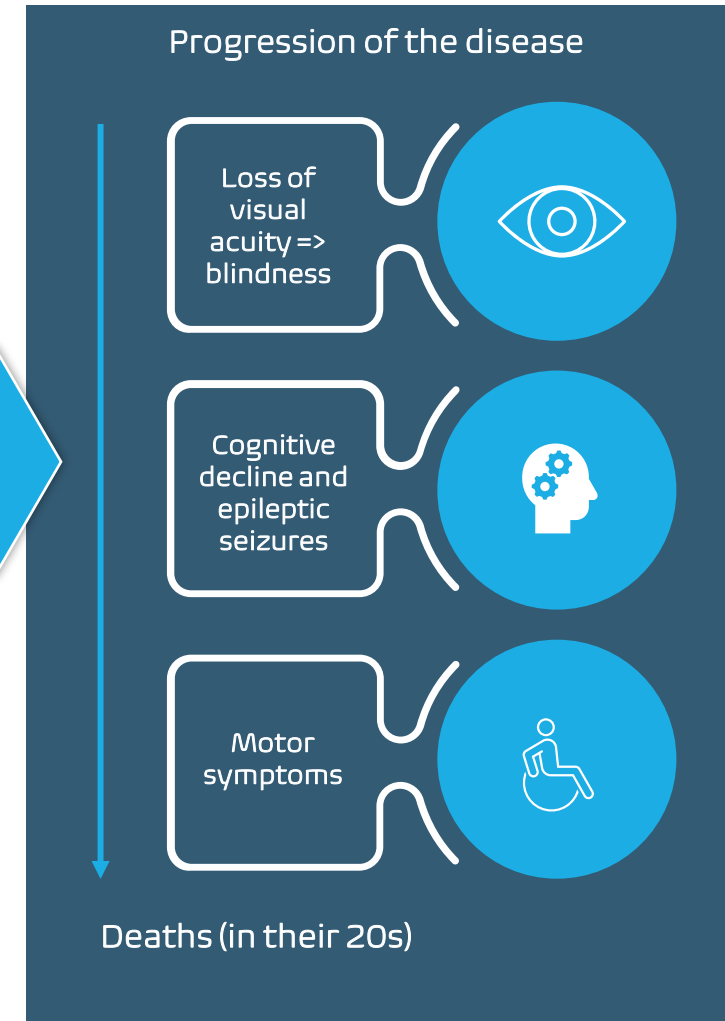
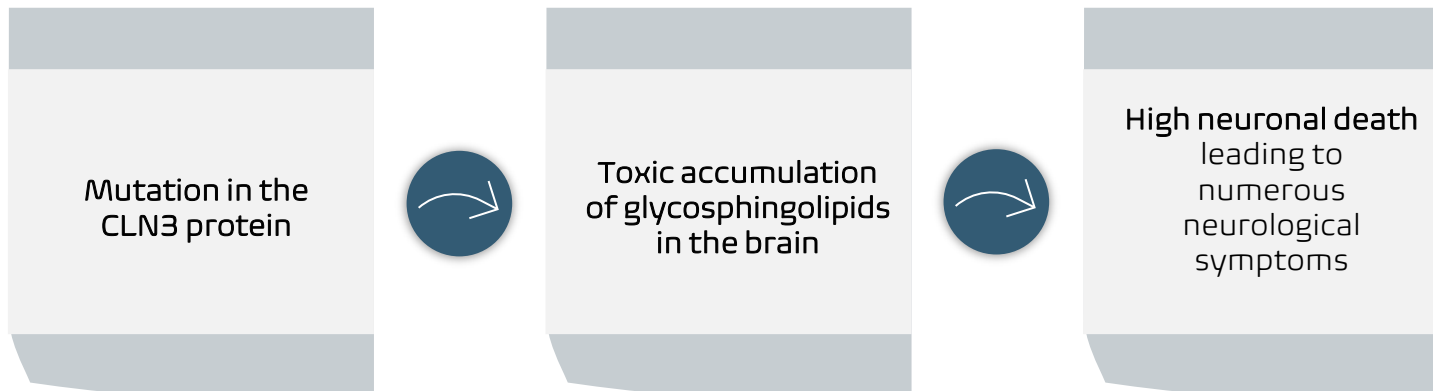


No treatment registered



Autosomal recessive transmission, founder effect localized in the Nordic countries

## Scientific rationale that led to Batten-1: how to reduce glycosphingolipids accumulation?



<sup>1</sup> On insurance database (Decision Resources Group)

<sup>2</sup> Orphanet data

<sup>3</sup> Work carried out by the laboratory of Emyr Llyod Evans at Cardiff University (funded by the Beyond Batten Disease foundation) and confirmatory work carried out by Theranexus

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

Blockage of GSL synthesis by miglustat (active ingredient in Batten-1), leading to a reduction in their accumulation

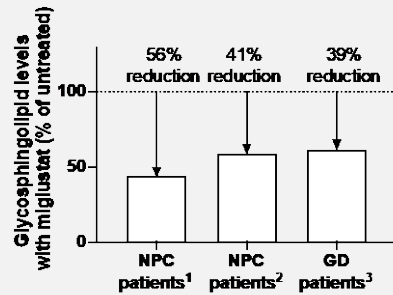
Activity already validated in humans in other indications

Reduction of cell death in the brain, with the objective of blocking disease progression

Significantly reduced GSL levels  
(clinical demonstration in similar indications)

Miglustat (the active agent in Batten-1) has already proven to be clinically effective in reducing GSL levels in two indications:

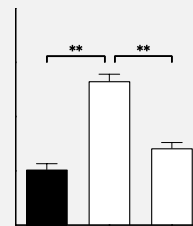
- Gaucher disease
- Niemann-Pick disease type C



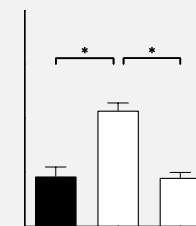
<sup>1</sup> Lachmann et al, 2004  
<sup>2</sup> Fan et al, 2013  
<sup>3</sup> Zavesca labeling package, EMA, 2005

Strongly reduced accumulation of GSL in CLN3

Ganglioside GM1



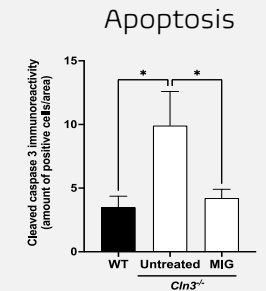
Globoside Gb4



Cellular models CLN3<sup>1</sup>



Normalized apoptosis in CLN3



CLN3 vivo models<sup>1</sup>



Batten-1 is effective in targeting glycosphingolipid synthesis and normalizing apoptosis<sup>1 2</sup>

<sup>1</sup>Work carried out by the laboratory of Emyr Llyod Evans at Cardiff University (funded by the Beyond Batten Disease foundation) and confirmatory work carried out by Theranexus

<sup>2</sup>Glycosphingolipid reduction with miglustat as a therapeutic strategy for CLN3 and other neuronal ceroid lipofuscinoses, Dr Emyr Lloyd-Evans, Cardiff University

# 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 swallowing difficulties.
    - ▶ 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 and 2022).
- Orphan Drug Designations granted by the FDA (USA) and the EMA (EU)



# FDA approval on efficacy endpoints and design of Phase 3 study

(our press release dated May 9<sup>th</sup> 2023)



- **Randomized double-blind study** evaluating the efficacy of Batten-1 in 2 parallel groups over a 2-year treatment period, versus placebo
- Target population: pediatric population of approximately 60 patients aged 4 to 16 years
- **Additional cohort of 9 open-label patients** recruited in parallel, evaluated on the same criteria as the main cohort of approximately 60 patients

The main endpoint of phase 3 will be visual acuity with, as secondary endpoints, the evaluation of cognitive and motor functions

## Visual acuity



Evaluated by:

- Either a quantitative LogMar<sup>1</sup> scale
- or a qualitative scale in the most affected patients, for whom quantitative assessment is not possible

## Cognitive functions



- Measured by the Verbal Comprehension Index from the Wechsler Intelligence Scale for Children according to age

## Motor functions

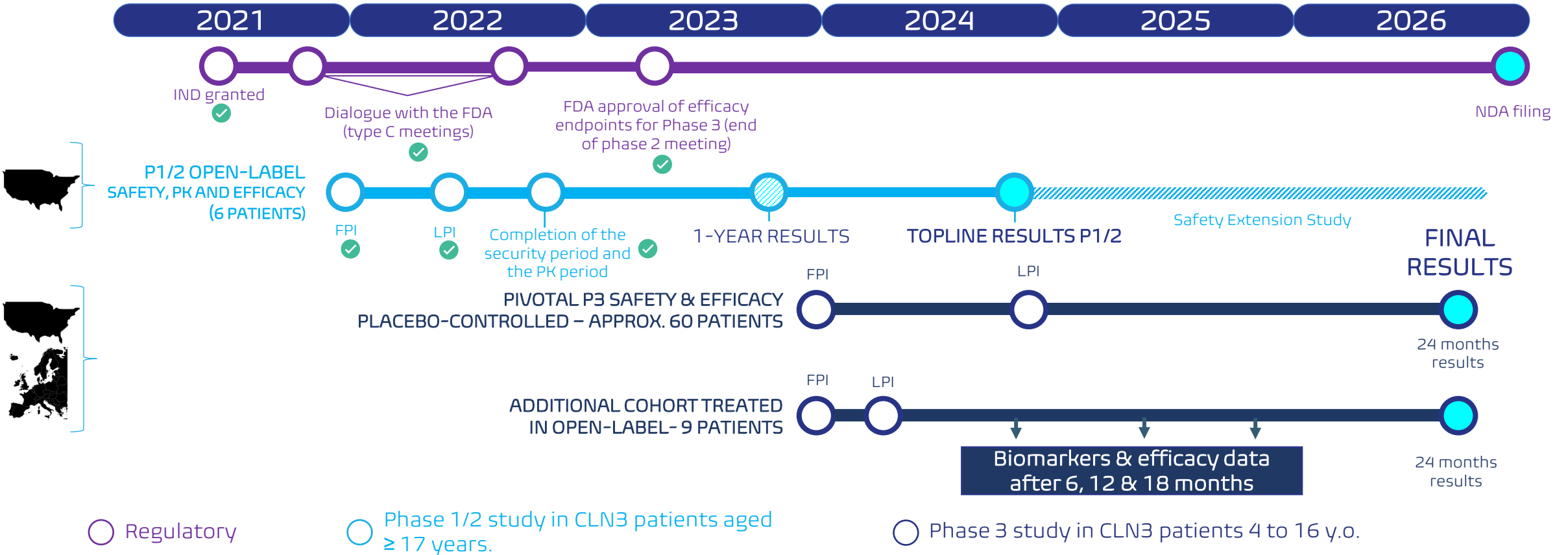


Evaluated by:

- a selection of motor items from the Unified Batten Disease Rating Scale (UBDRS),
- visual functions, assessed by OCT scan



# Development plan with Phase 3 results by the end of 2026 followed by market approval



➤ Final results by the end of 2026 followed by approval (notably in the US & Europe)

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

Territories	Number of currently diagnosed patients	Sales based on diagnosed patients (USD m) <sup>1</sup>	Estimated prevalence <sup>4</sup>	Sales based on est. patients (USD m) <sup>1</sup>
United States	~750 <sup>2</sup>	~480	~1 200	~765
EUS (Germany, France, Italy, Spain, United Kingdom)	~340 <sup>3</sup>	~50	~500	~75
Nordic countries	~120 <sup>3</sup>	~15	~180	~20

**MARKET OPPORTUNITY**

**\$545 Mn**

**\$860 Mn**

1: Price estimates based on interviews with payers and KOLs in Q1 2022 (source: report by Justin Stindt Consulting for Theranexus).

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

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

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

# Theranexus: a strong value creation opportunity for investors

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

- ✓ Batten-1 P3 results in 2026
- ✓ A well understood and effective mechanism
- ✓ A high degree of protection (proprietary formulation + ODD)
- ✓ Easy access to a high potential market (>\$500M/year):
  - Partnership already established with the BBDF (the largest CLN3 patient association in the world)
  - An optimized process until approval
  - Limited number of prescribers and the potential for early access to accelerate treatment adoption
- ✓ No treatment on the market and no competitor in active development

## An R&D platform dedicated to rare neurological diseases:

- ✓ Platform based on very strong scientific collaborations
- ✓ A new ASO candidate<sup>1</sup> in the second half of 2023

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# Appendix

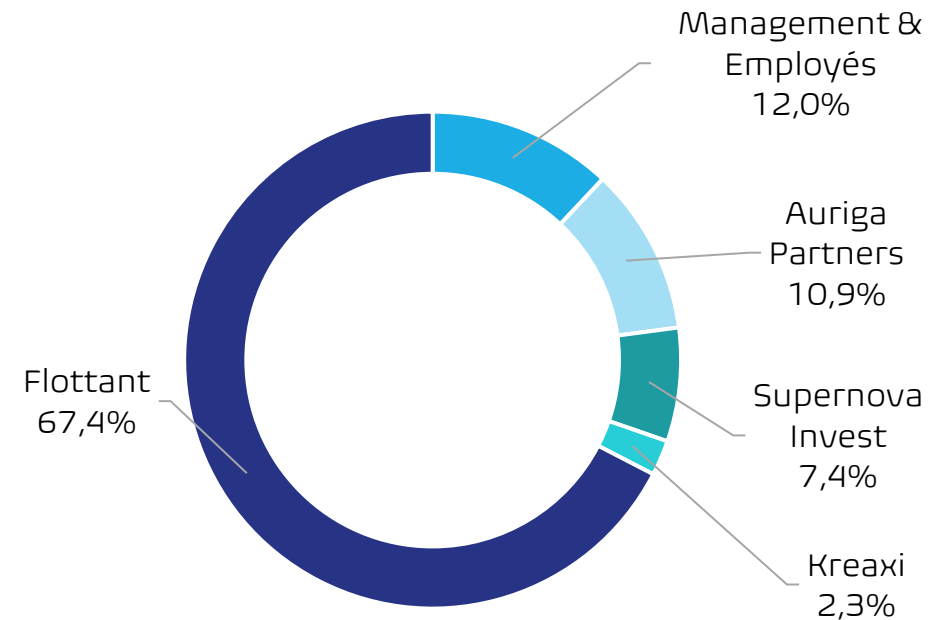
# Listing and shareholding

## Financial data

- ISIN : FR0013286259 - Mnemo : ALTHX
- Market: Euronext Growth
- Share price on May 5<sup>th</sup>, 2023: €1.20.
- Market capitalization: 6.388 million euros
- Coverage: ODDO BHF, Portzamparc
- Liquidity contract: Portzamparc

## Capital structure

Number of shares: 5,314,434



# Simplified income statement

## Controlled expenses

In K€ (French standards)	2022	2021
Operating income	1,246	25
Other purchases and external expenses	3,967	5,591
Salaries and social charges	3,165	2,689
Depreciation and amortization	332	1,204
Other expenses	39	53
<b>Operating result</b>	<b>(6,257)</b>	<b>(9,512)</b>
Financial result	(158)	(396)
Extraordinary expenses	(771)	-
Income tax	619	1,758
<b>Net income</b>	<b>(6,567)</b>	<b>(8,150)</b>



Cash and cash equivalents as of March 31, 2023: €7,300K

# THANK YOU FOR YOUR ATTENTION!

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