INVESTOR PRESENTATION

Theranexus

LABORATOIRE EN 1ÈRE LIGNE
CONTRE LES MALADIES
NEUROLOGIQUES

YOUR CONTACTS

Franck MOUTHON

Co-founder and Chairman and CEO

- Franck Mouthon holds a degree in life sciences from the École Normale Supérieure
- Joined the Life Sciences Department of the French Alternative Energies and Atomic Energy Commission (CEA) in 1995
- Founded CEA spin-off Theranexus in March 2013 with Mathieu Charvériat
- Board member of France Biotech

Thierry LAMBERT

CFO

- Thierry Lambert holds a degree in business administration from Birmingham University and an MBA from INSEAD
- 4 years of experience in syndicated and corporate finance
- 5 years as Chief Financial Officer for listed companies Naturex and then Safe Orthopaedics
- Joined Theranexus in 2017

Theranexus

A DISRUPTIVE BIOTECH IN CENTRAL NERVOUS SYSTEM RESEARCH

MAJOR ASSETS

A high-potential diversified portfolio

Phase 2 for Parkinson's disease
Two other high-need indications:
Alzheimer's disease and neuropathic pain
Potential for blockbuster technologies
Shortly Phase I-III in Batten disease

A POWERFUL TOOL

A unique & versatile platform for generating drug candidates

First platform based on neuron-glia interactions

DIVERSIFIED PIPELINE

Clinical trials

	Candidate selection	Pre-clinical trials	Phase I	Phase II	Phase III		
THN 102 (BREVET DEPOSE)			**Ph2 results H2-2019**			Excessive Daytime Sleepiness linked to Parkinson's disease **No treatment to date**	
THN 102 (BREVET DEPOSE)			**Ph2 definitive results Q1-2019**		**Program viability under assessment**	Narcolepsy **$2 billion**	
THN 201 (BREVET DEPOSE)			**Ph1b results H2 2019**			Neurocognitive disorders linked to Alzheimer's disease **$3.2 billion**	
THN 101 (BREVET DEPOSE)				**Ph2 under preparation**		Neuropathic pain **$3 billion**	
BBDF 101 (BREVET DEPOSE)				**Ph1-3 (pivotal) under preparation**		Batten disease * **No treatment to date**	
THN xx						New indications	

*All figures are derived from Datamonitor reports (ND, dementia); company annual reports (Jazz Pharmaceuticals, Teva)

THERANEXUS PLATFORM: PROPRIETARY, SCALABLE & VERSATILE

CNS DRUGS

DRUG SEEN AS THE 1ST LINE-TREATMENT

Condition with a strong unmet need for improved efficacy (with the current arsenal of therapeutics)

CNS drugs 1st line- treatment for CNS conditions*

Action on the neuron

GLIAL CELL MODULATOR

DRUG REPOSITIONED AS A MODULATOR

Optimization of the glial network

Theranexus library of 27 glial cell modulators

PATENTED

THN XXX

3 major advantages

- **Ambition to achieve superiority at all stages (*Best in class*)**
- **New monopoly on use (patent)**
- **Higher probability of success, greater flexibility and shorter time-to-market**

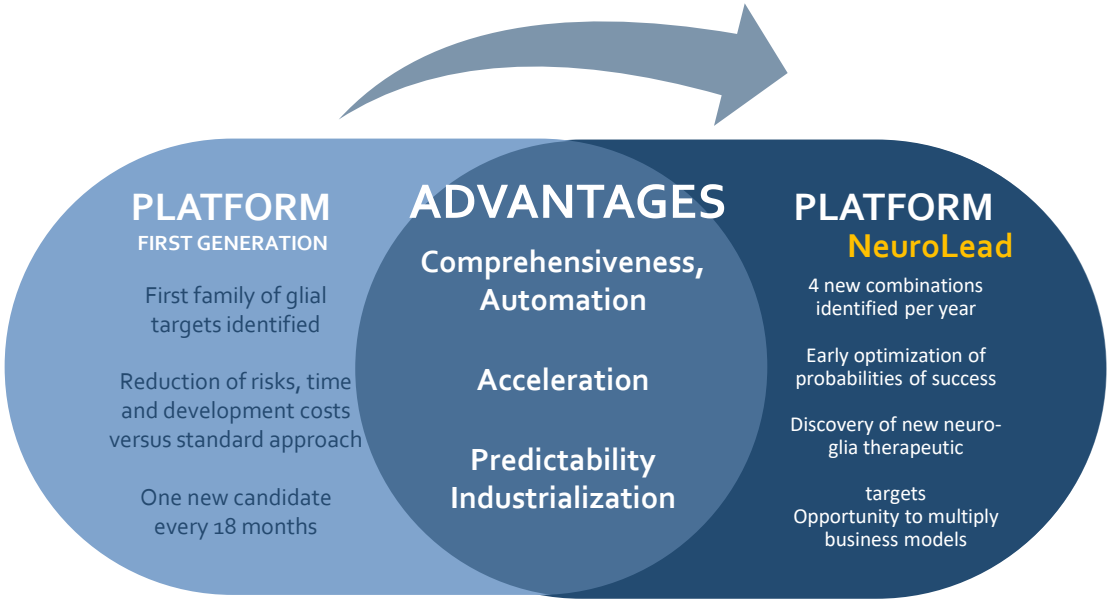
*Central Nervous System

NEUROLEAD :STRENGTHENING THE LEAD GENERATION PLATFORM

NeuroLead

- Development of a drug candidate generating platform based on neuron-glia interactions
- Prestigious partners:
- Capacity to build on the latest innovations in neuroscience and Deep Learning
- Funding package of €6.2m from BpiFrance, for the consortium managed by Theranexus

A NEW PLATFORM FOR DRUG CANDIDATE GENERATION FOCUSED ON MEDICAL AND INDUSTRIAL VALUE



FROM PIONEER TO REFERENCE PLAYER IN NEUROLOGY

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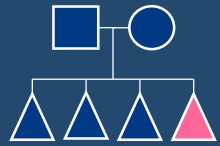
Batten disease or juvenile neuronal ceroid lipofuscinosis (NCL3) – A rare genetic disease that is fatal between the ages of 20 and 30

EPIDEMIOLOGY AND PHYSIOPATHOLOGY OF NCL3

c. 3,000 patients (all NCL types)



Autosomal recessive

Diagnosis in children aged 4 to 8

Blindness

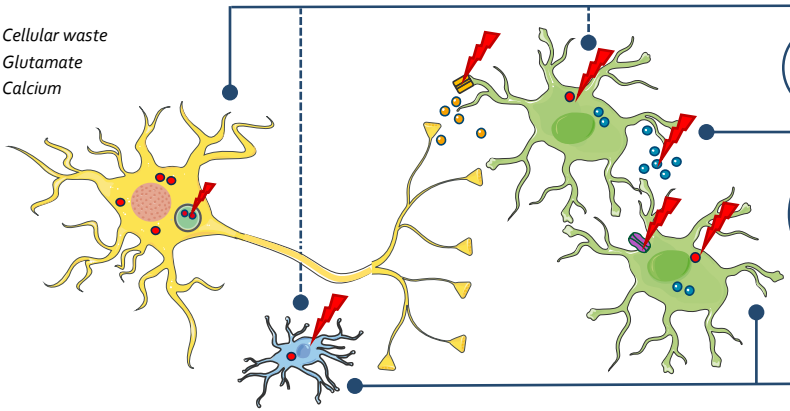
Cognitive decline

Loss of motor skills

No registered treatment

ROLE OF NEURON-GLIA INTERACTION IN NCL3

- *Cellular waste*
- *Glutamate*
- *Calcium*



Lysosomal and autophagic signaling pathway deficiency

Defective glutamate recycling pathway
Disruption of calcium pathways
Dysfunctions in hemichannel activity

Neuroinflammation

ASTROCYTE FUNCTIONS ARE SEVERELY IMPAIRED, LEADING TO THE NEURONAL DEFICIENCY RESPONSIBLE FOR THE SYMPTOMS

The product – BBDF 101 - miglustat-trehalose combination

FOUNDATION

BEYOND BATTEN DISEASE FOUNDATION

Created in 2008 by Craig Benson
USD 30 million R&D investment leading to identification of **BBDF101**

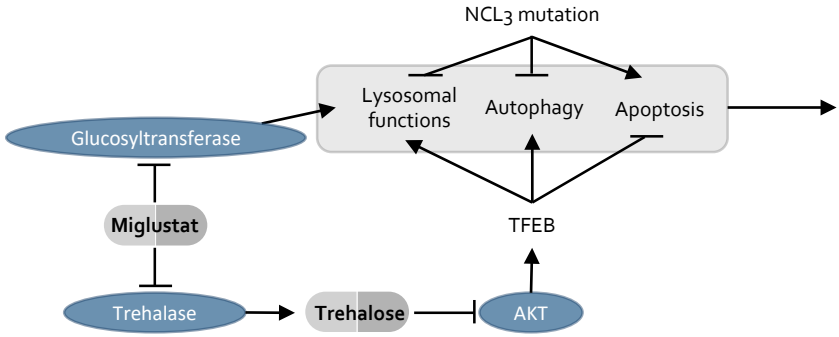
BBDF101 **Trehalose + Miglustat combination**

Pre-IND meeting – Strong support from FDA

- Eligible for **Fast Track** procedure (MA in 6 months)
- Eligible for **pediatric ODD** Exclusivity for 7.5 years in USA and 12 years in EU
- Patent grant underway in USA, valid until 2036

→ Ready to enter Phase I/III , pre-registration, open label, **against historical data**

MECHANISM OF ACTION



PRECLINICAL DEMONSTRATION

NCL3 transgenic mice

- ↑ Cell survival
- ↓ Neuroinflammation
- ↓ Lysosomal signaling pathway deficiency

STRONG IMPACT ON MECHANISMS RESPONSIBLE FOR NCL3 SYMPTOMS

Competitive environment and market opportunity

COMPARABLES

ZAVESCA 100 (miglustat) capsules	Myozyme (alglucosidase alfa)	elaprase (idursulfase)	Brineura (cerliponase alfa)
6,000 cases USA 5,000 cases EU	5,000 cases USA 1,800 cases EU	500 cases USA 400 cases EU	500 cases USA 250 cases EU
Gaucher disease	Pompe disease	Hunter syndrome	NCL2
$240,000/yr/patient €55,000/yr/patient	$300,000/yr/patient	$375,000/yr/patient	$700,000/yr/patient
Peak (2014): $113m	Peak (2018): $947m	Peak (2018): $634m	Peak (2027): $359m (f)

Notes: All drugs have 'Orphan Drug Designation' status and Brineura obtained a pediatric voucher (sold for $120m)

COMPETITION IN CLINICAL DEVELOPMENT

NCL3 AAV9 **gene therapy**
Amicus Therapeutics

Phase I/II: Recruitment underway

Duration: 36 months' control
Completion due in December 2022

Design: n=7

MARKET ACCESS

Access to patients highly structured – Direct sales force of limited size

USA: Two main associations (BBDF and BDSRA)
and 18 hospitals taking care of Batten patients
EU: 7 primary centers (France, UK, Germany, Norway)

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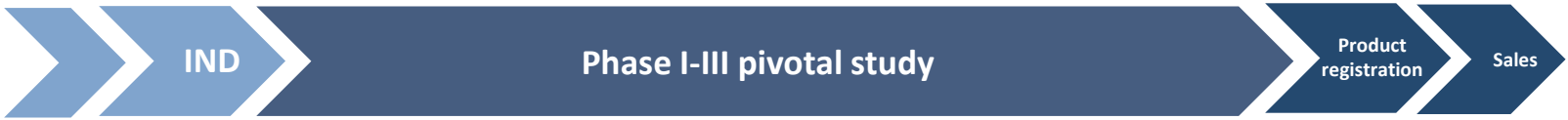
BBDF 101 development plan

Signing of firm agreement between BBDF and Theranexus (exclusivity of negotiations in place for 6 months from signing of Term Sheet)

Application for IND (authorization to enter clinical trial phase) from the FDA (US Food and Drug Administration)

Registration of product – the FDA has already confirmed that the product would be eligible for fast-track registration (rare pediatric disease)

Theranexus plans to market the product directly. The limited number of specialist doctors and therefore of potential prescribers limits the required marketing expenditure.

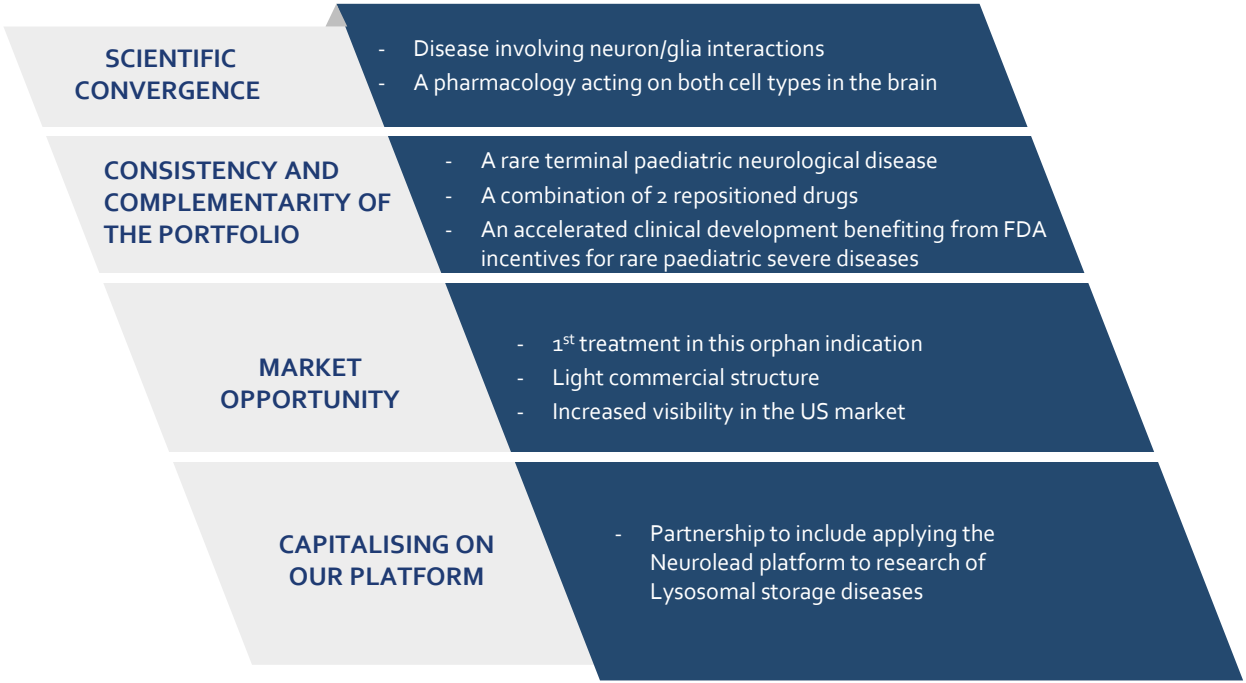


2019 2020 2021 2022 2023 2024

Clinical trials:

- Phase I-III (leading directly to product marketing)
- On 25 patients in the USA
- Open label
- The evaluation is based on comparing the disease progression in patients recruited for the trial against the natural course of the disease as described by several existing groups of NCL3 patients – similar to the trials conducted by Biomarin for Brineura™
- Trial duration: 30 months (+ 6 months of recruitment)

Strategic interest of BBDF101 for Theranexus



- **Term Sheet signed with 6-months exclusivity**
- **Theranexus will market the product**
- **The foundation will have an interest in the additional value created in the form of milestones and royalties on future sales**

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THN102: A DRUG CANDIDATE TO COMBAT SLEEPINESS RELATED TO PARKINSON'S

MODAFINIL | FLECAINIDE

THN 102

Parkinson's disease

Sleepiness, attention, cognition

Almost **1 million patients** (G7)
30-50% of Parkinson's sufferers

NO TREATMENT

No drug approved to date
6.1 million Parkinson's sufferers[1] – 40%[2] affected by sleepiness…

4 drug candidates are at a clinical stage, all targeting only neurons

Phase II clinical trial underway

Design:
Double-blind study comparing 2 doses of THN102 with a placebo in a three period cross-over format: each patient receives either THN102 or the placebo, chosen at random, over three periods of 2 weeks

Efficacy criteria: sleepiness, attention, alertness, cognition

Study carried out on **60 Parkinson's patients** (including one group in the United States)

Update at 17/04/2019

More than half of patients recruited

IND authorization for clinical trial in the United States

Eligible for 505(b)(2) regulatory pathway (opportunity to benefit from existing data for reference drugs)

Results expected in H2 2019

[1]: European Parkinson's Disease Association
[2] Market research study performed by LSA Partnering & Analytics

THN201: A HIGH-POTENTIAL CANDIDATE FOR DEMENTIA

DONEPEZIL | **MEFLOQUINE**

THN 201

Neurocognitive disorders linked to **Alzheimer's disease**

Impaired memory, reasoning and orientation

15 million patients in 2015 (G7)
19 million by 2030
45% of patients undiagnosed

DONEPEZIL

$3.2 billion
(annual cost of treatment per patient €4,000-5,000)

23 drug candidates at clinical trial stage

Launch of Phase Ib clinical trial

Under the CX-COG project funded by the French **"Fonds Unique Interministériel"** (FUI AAP22)

Double-blind randomized study comparing placebo and standard of care drug (Donepezil)

Trial conducted on three parallel groups evaluating the cognitive activity, tolerability and pharmacokinetic profile of THN201

Key efficacy criteria:
measurement of pro-cognitive activity through a scopolamine test

Trial conducted on **150 healthy volunteers** in a parallel group design **in 8 centers in France**.
Repeated dose treatments to be orally administered once a day over 15 days.
Update at 17/04/2019:
more than 40% of volunteers recruited

Results expected in H2 2019

THN101: DRUG CANDIDATE READY FOR PHASE II TRIALS: PAIN

AMITRIPTYLINE | **MEFLOQUINE**

THN 101

Neuropathic pain	Preparation stage for Phase II clinical trial
Chronic pain with occasional stabbing pain, sensations of burning or electric shocks	**Key efficacy criteria:** pain scale
70 million patients (Europe, USA, Japan)	**Double-blind randomized study comparing placebo with standard of care drug** (Amitriptyline)
AMITRIPTYLINE	**Trial conducted on three parallel groups:** Amitriptyline 25 mg/day and mefloquine 10 mg/day vs. Placebo and vs. active comparator (amitriptyline). Regular evaluation of pain and analysis of multiple secondary markers and tolerability.
$3 billion (annual cost of treatment per patient $3,000-4,000)	Patients suffering from neuropathic pain caused by diabetes or post-herpetic neuralgia (following shingles)
32 drug candidates at clinical trial stage	Multi-center international trial conducted on **370 patients** Conducted in parallel at **40-45 centers in Europe**.

Phase II trial program at preparation stage

THN102: PARTNERSHIP STRATEGY FOR THN102

Candidate selection | Pre-clinical trials | Clinical trials: Phase I — Phase II — Phase III

THN 102 — BREVET DÉPOSÉ

Market and dimension

Excessive Daytime Sleepiness linked to Parkinson's disease
No treatment to date

Specialists in EDS or CNS

Jazz Pharmaceuticals
SK biopharmaceuticals
Sumitomo Dainippon Pharma
Zambon
Lundbeck
ucb Pharma

Generalists and "big pharma"

Takeda
NOVARTIS PHARMACEUTICALS
Boehringer Ingelheim
Mitsubishi Tanabe Pharma

MULTIPLE OPPORTUNITIES FOR PARTNERSHIPS:

+ INTRINSIC COMMERCIAL POTENTIAL OF PRODUCT

+ OPTIMIZATION OF SALES FORCES USED FOR PARKINSON'S

+ POSSIBILITY TO REACH NEW MARKET FOR EDS SPECIALISTS

BLOCKBUSTER POTENTIAL FOR AN INDICATION WITH A GROWING BUT UNTREATED NEED

STRONG INTEREST AMONG INDUSTRY PLAYERS FOR THE FIRST 3 CONDITIONS TARGETED

CONDITION	DATE	SELLER	BUYER	PROFILE	DEVELOPPEMENT STAGE	UP FRONT (M$)	MILESTONES (M$)	ROYALTIES (M$)
Parkinson's disease	2018	Prexton	Lundbeck	NCE	Phase II	**123**	**993**	-
	2016	Cynapsus	Sunovion	LCM	Phase III	**624**	-	-
Neuropathic pain	2015	Convergence	Biogen	NCE	Phase II	**200**	**475**	NC
	2015	Spinifex	Novartis	NCE	Phase II	**200**	**500**	NC
Alzheimer's disease	2017	Lyndra	Allergan	LCM	Préclinique	**15**	**90**	NC
	2016	Chase Pharma	Allergan	Combination	Phase I/II	**125**	**875**	NC
Other neurological disorders	2014	Avanir	Otsuka	Combination	Market	**3,500**	-	-

TURNING POINT IN VALUE AT THE END OF PHASE II
(BETTER RATIO OF DEVELOPMENT COSTS
TO IMMEDIATE AND SUBSEQUENT REVENUES)

[1] *New Chemical Entity*

[2] *Life Cycle Management*

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2018 P&L

In K€ (French GAAP)	2017	2018
Operating income	**164**	**175**
Other purchases and external charges	1 477	4 969
Salaries and benefits	1 370	2 117
Depreciation and amortization	26	55
Other operating expenses	11	24
Operating result	(2 719)	(6 990)
Net financial income	(126)	(31)
Corporate tax	730	1 721
Net income	(2 115)	(5 301)

GOOD CONTROL OVER EXPENSES IN A CONTEXT OF ACCELERATED CLINICAL DEVELOPMENTS

MAINLY RESEARCH TAX CREDIT

CASH AS AT MARCH 31ST 2019: 12,4M€
+R&D TAX CREDIT RECEIVABLE: 1,7M€

INVESTORS RELATIONS AND SHAREHOLDERS

DATA AS AT 26 JUNE 2019

ISIN : FR0013286259 - **Mnemo:** ALTHX

Listed on Euronext Growth

Stock price : 5,3€ (on Jun. 18th 2019)

Market Cap : 16 M€

Liquidity contract : Portzamparc

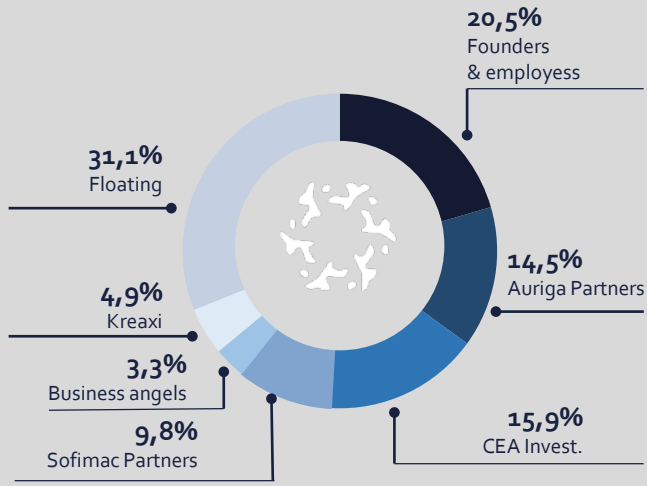
Cash as at March 31st 2019 : 12,4 M€

ALTHX EURONEXT GROWTH

ENTREPRISE INNOVANTE bpifrance

ELIGIBLE PEA PME

SHAREHOLDERS



PUBLICATION SCHEDULE FOR 2019

P2 results: H2-2019

THN 102

P1b results: H2-2019

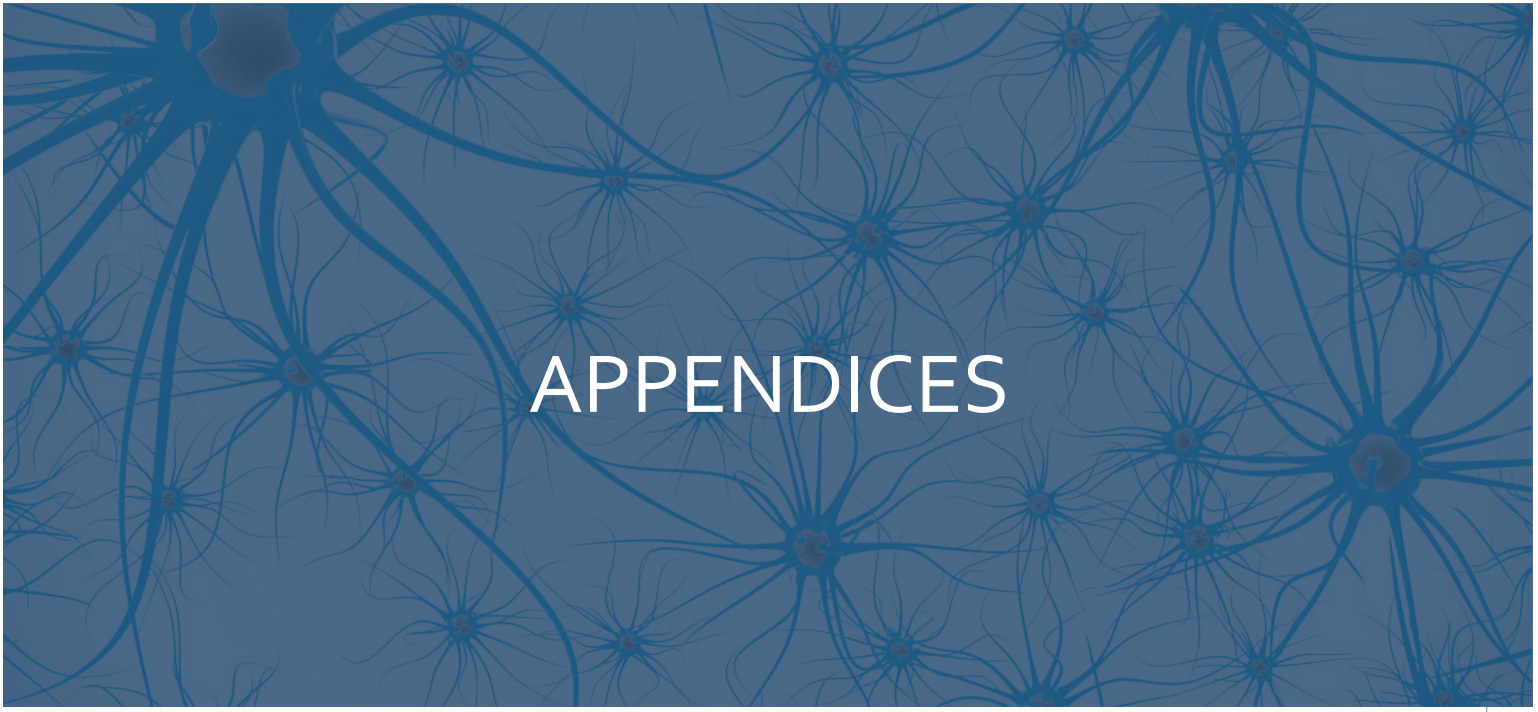
THN 201

Results of P1a: end of H1-2019
Preparation stage of P2: pain H2-2019

THN 101

IND for BBDF 101 in Batten disease

BBDF 101



APPENDICES

Clinical development – Example: Brineura (BioMarin) for CLN2

CLN2
500 CLN2 cases US – 250 cases EU
Diagnostic between 2 and 4 years old
Autosomal recessive
Dementia, motor deficit,
vision loss
Mortality between 8 and 12 years old

Phase I/II Pre- registration
Duration: **2 years** –extension study on going
n=23 patients [Enrollment in six months]

ICV Administration – 4h30
infusion every two weeks

Natural history control group
DEM-CHILD database –
Comparing the language and
motor scores

Price: US$ **700 000** /year/patient
MAA 2017, Revenue 2027(f) US**$ 359 M**
Priority Review Voucher ~ US**$ 100 M**

ODD in Europe and in the US
European MAA; FDA approval

BioMarin – pioneer in enzymatic therapy for rare genetic diseases
Market Capital: US$ 15 B (Nasdaq)

TRANSFORMING RESEARCH INTO INNOVATION

PRINCIPLE:
Enhance neuron action with the modulation of glial cells

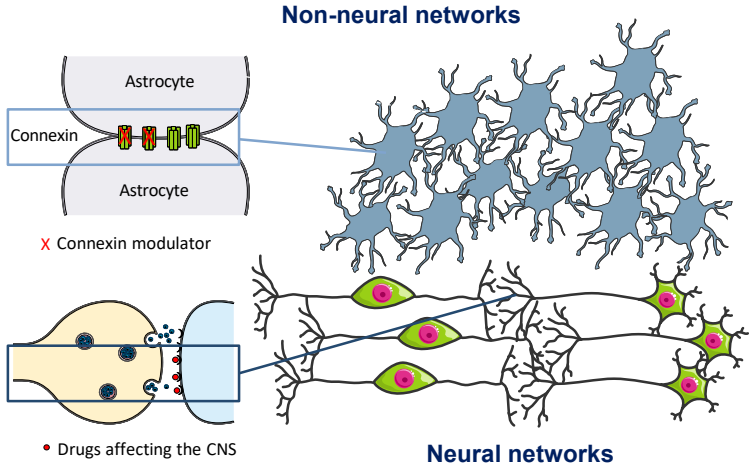
APPLICATION:
Combine medication that targets neurons with a medication that optimizes neuroglial interaction

Connexin modulator

CNS drug

(Psychostimulant, antidepressant, anxiolytic, etc.)

Action on neurotransmitter systems

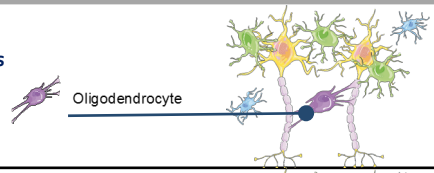
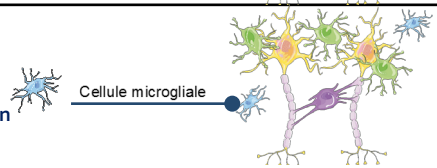
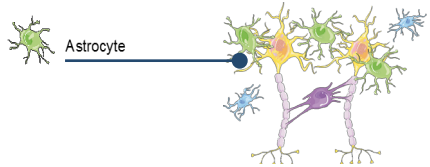
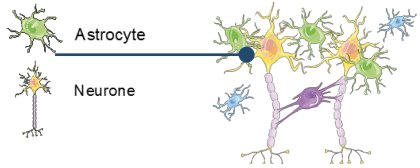


The modulation of glial connexins optimizes the neuroglial interface to **improve the way in which neurons react to CNS drugs**

Giaume et al., Nat Rev Neurosci, 2010
Rouach et al., Science, 2008
Picoli et al., J Biomol Screen, 2012
Duchêne et al., Sleep, 2016
Charvériat et al. Front Cell Neuro, 2017

THE CHALLENGE: MAXIMISE NEURON RESPONSE TO EXISTING DRUGS BY TARGETING THE ENVIRONMENT

TARGETING GLIAL CELLS : A SOARING APPROACH

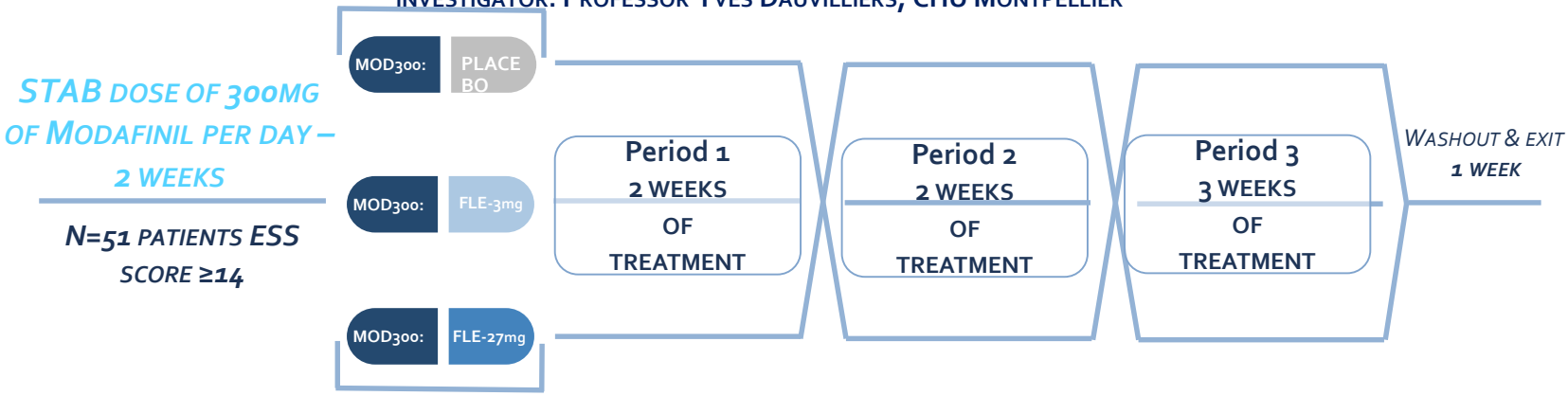
APPROACH	MECHANISM OF ACTION	SOCIETIES
Oligodendrocytes & remyelination		Biogen, ACØRDA THERAPEUTICS, astellas
Microglia & neuroinflammation		DENALI THERAPEUTICS, Daiichi-Sankyo, IONIS PHARMACEUTICALS
Astrocytes & metabolism		Astrocyte Pharmaceuticals Inc., NEURACLE SCIENCE, GliaPharm, GliaCure
Interactions neurons / Glia		Theranexus

Phase IIa trial: NARCOLEPSY

SAFETY AND EFFICACY OF THN102 IN SLEEPINESS IN NARCOLEPTIC PATIENTS

DOUBLE-BLIND MULTICENTER STUDY COMPARING 3 TREATMENTS OVER 5 SITES IN FRANCE AND ONE IN BELGIUM

INVESTIGATOR: PROFESSOR YVES DAUVILLIERS, CHU MONTPELLIER



Key efficacy criteria	Results	Discussion
THN102 sleepiness much less than Modafinil sleepiness	No significant statistical difference excellent tolerability profile	Patient sleepiness on Modafinil equivalent to patients without treatment[1] Too high proportion of patients not responsive to Modafinil in the study

(1): Thorpy et al. 2019 / (2): Bogan et al. 2015 / (3): Dauvilliers et al. 2013 / (4): Fry et al. 1998