

# PHARNEXT

16 janvier 2019, Hotel Bedford



**Biotech**  
AGORA



**PHARNEXT**

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# Vos interlocuteurs



**Olivier Legrand**  
Directeur  
Financier



**Rodolphe Hajj**  
Directeur de la  
Pharmacologie

# Un fondateur pionnier

Pr. Daniel Cohen

- Eminent **généticien, Daniel Cohen**
- Plus de **30 ans** d'expérience dans l'industrie biotechnologique et pharmaceutique
- Créateur de la **génétique moderne**
- Fondateur du **CEPH**, Centre d'Etudes du Polymorphisme Humain et du **Généthon**
- Découvreur de la **première carte du Génome Humain**
- Plus de **150 publications internationales**



# La Pleotherapy™ en deux mots

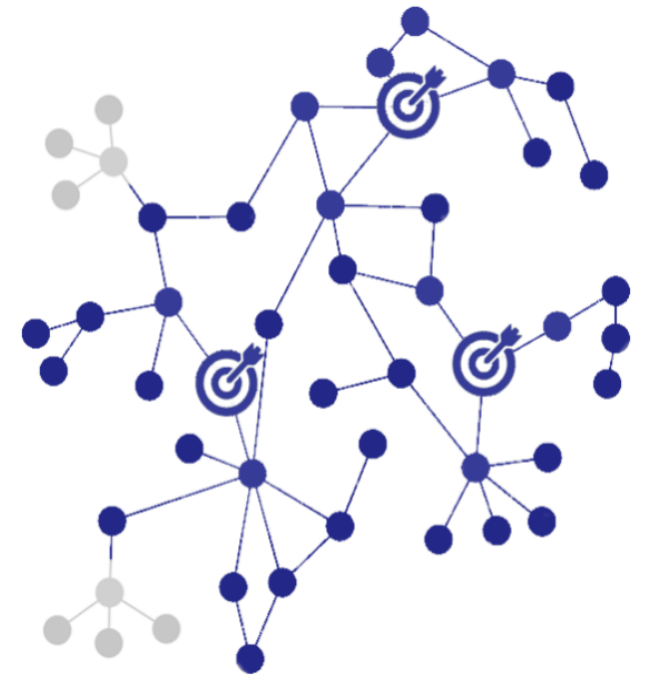
RESEAU DE LA MALADIE



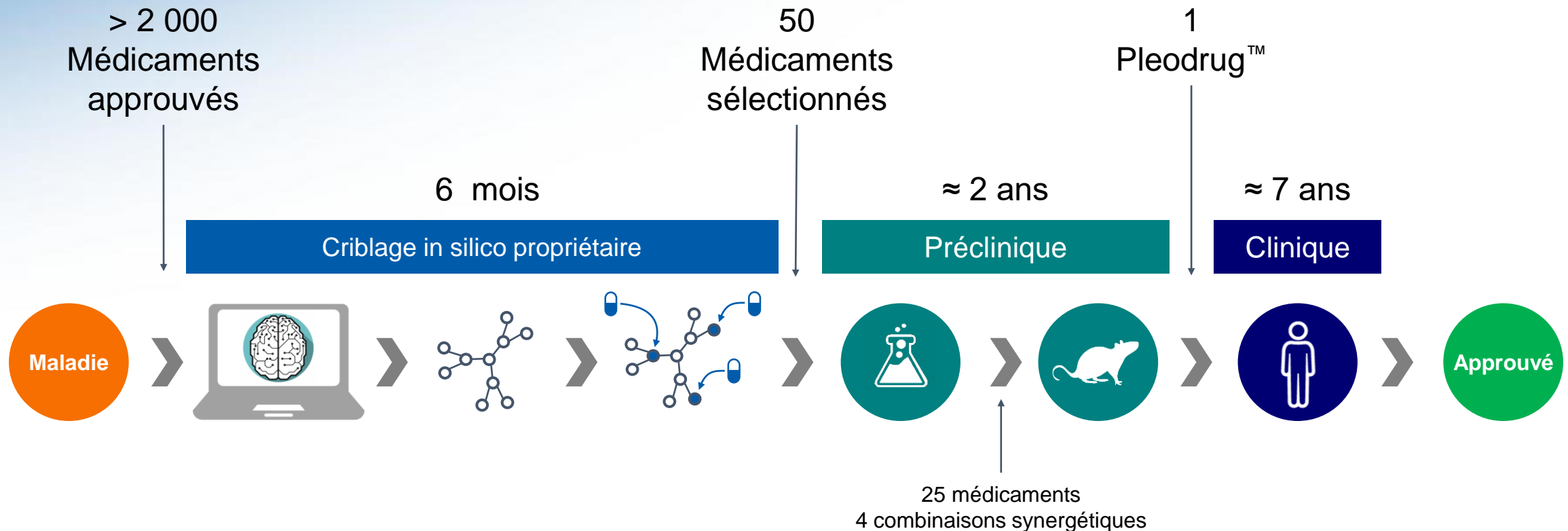
MONOTHERAPIE



PLEOTHERAPY™  
(Approche de Pharnext)



# Plateforme Universelle de R&D : Pleotherapy™



# La maladie de Charcot-Marie-Tooth de Type 1A

## SYMPTOMES

Neuropathie héréditaire débilante entraînant un handicap de la marche et des mains

## DIAGNOSTIQUE

Environ 50% de patients présentent des symptômes avant l'âge de 20 ans

## POPULATION

~100 000 personnes avec une CMT1A légère ou modérée aux USA et Europe

## OPTIONS DE TRAITEMENT

Aucun médicament approuvé, seulement des soins de soutien

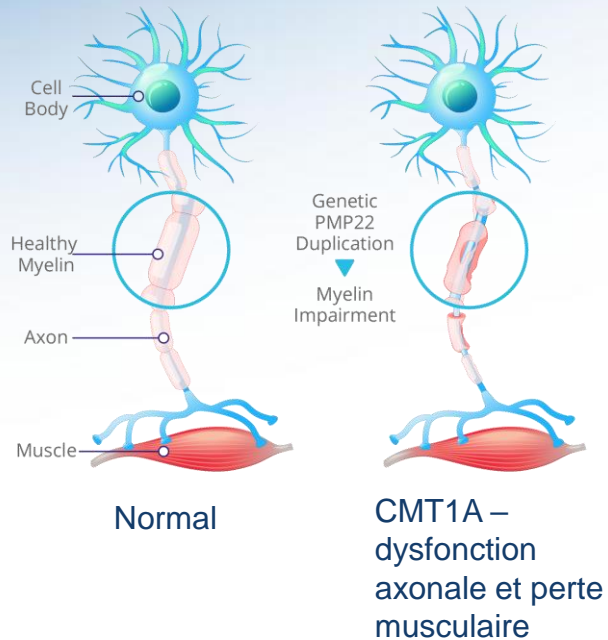
## BARRIERES A L'ENTREE

Brevets jusqu'en 2030. Statut de médicament orphelin accordé aux USA et en Europe

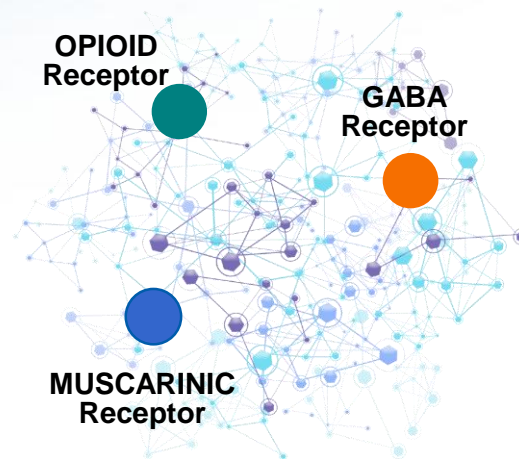


# Conception et mécanisme du PXT3003

## La maladie CMT1A en bref



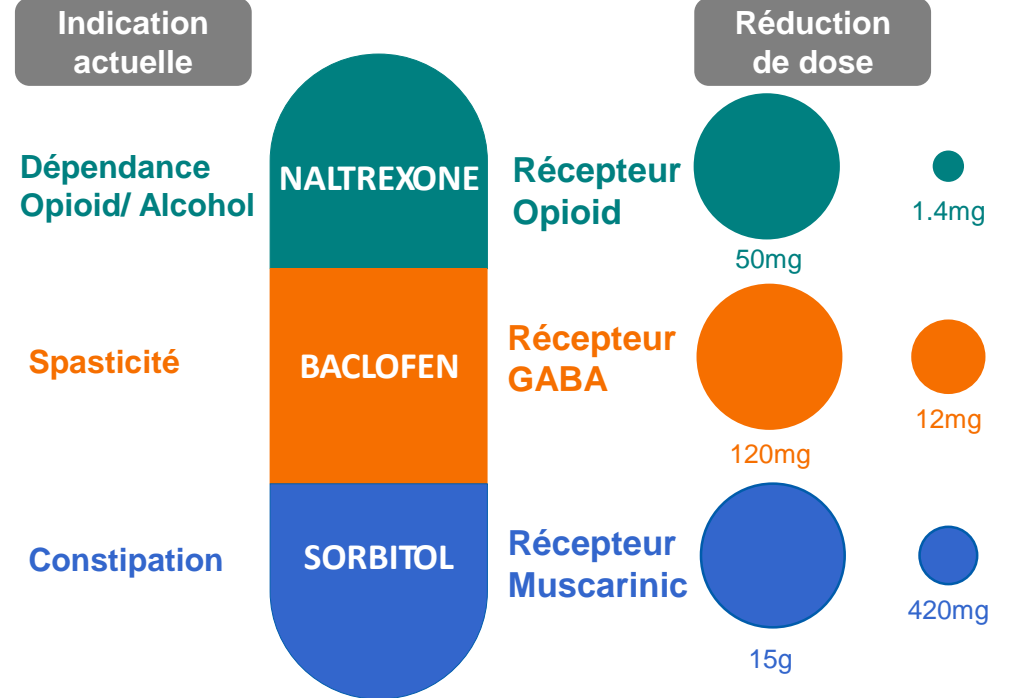
## Analyse du réseau



### Amélioration de :

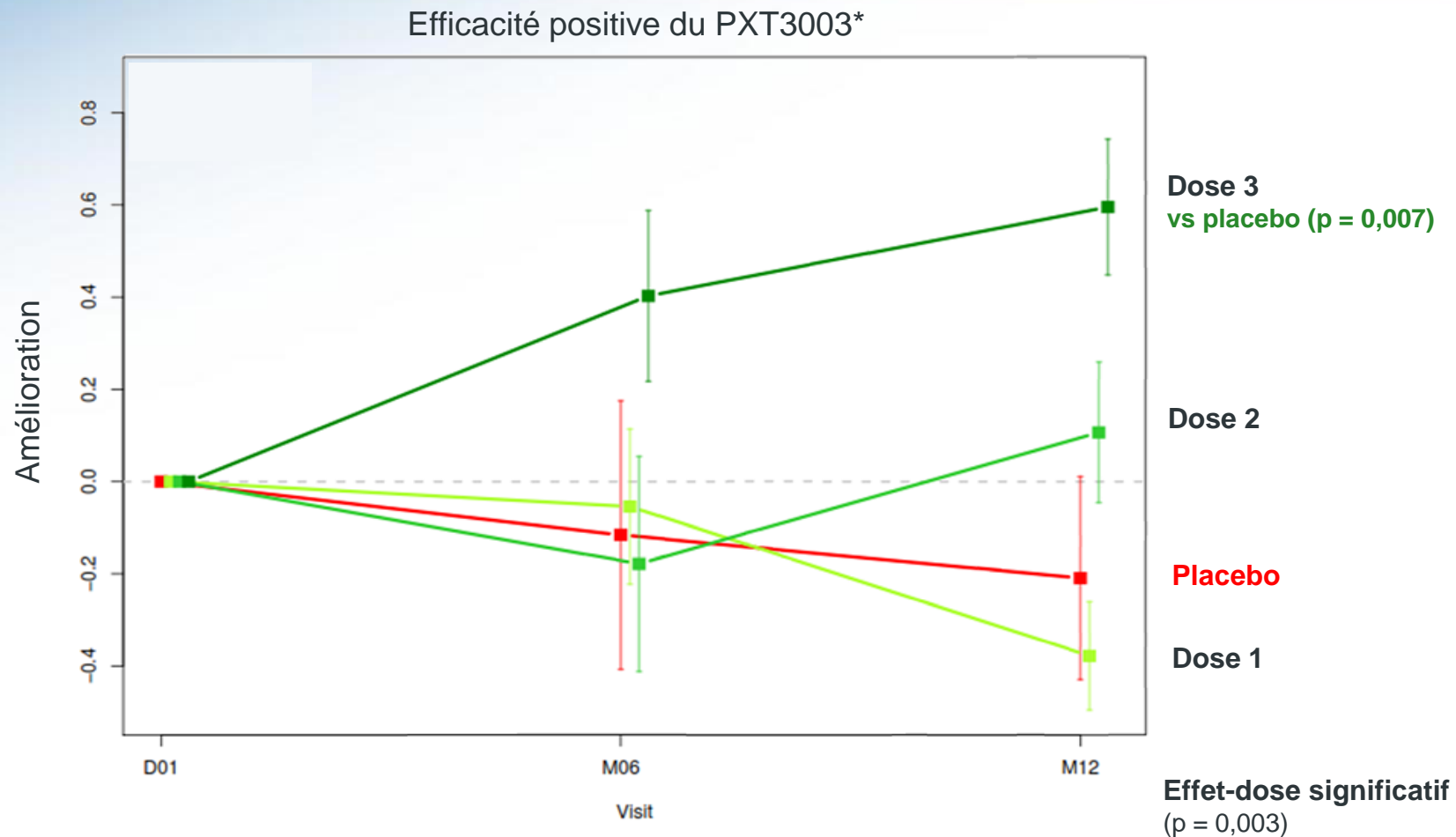
- Myéline
- Axone
- Muscle
- Inflammation

## Design du PXT3003





# Résultats positifs de l'essai de Phase 2 de PXT3003 dans la CMT1A



\*Toutes les mesures cliniques objectives et d'électrophysiologie agrégées en z-score.

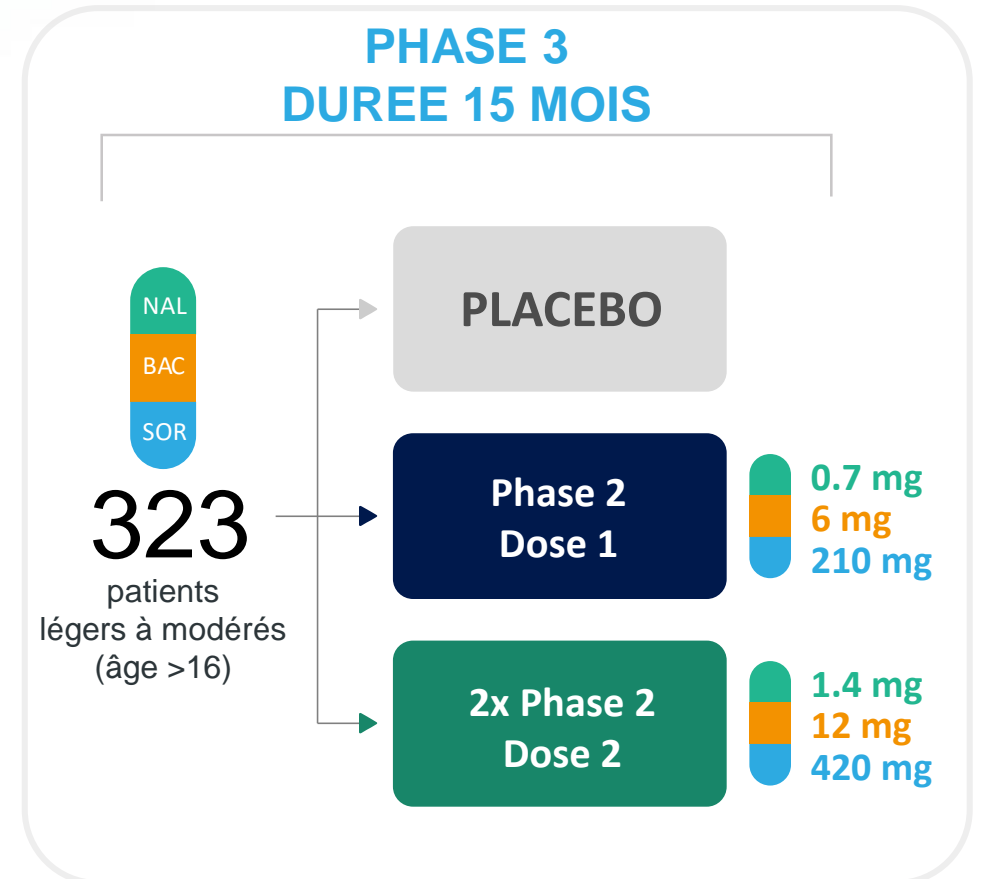
# Essai de Phase 3 dans la CMT1A

- **Critères d'évaluation définis avec les FDA & EMA**

- Critère principal : ONLS
- Critères secondaires : Cliniques moteurs et sensoriels

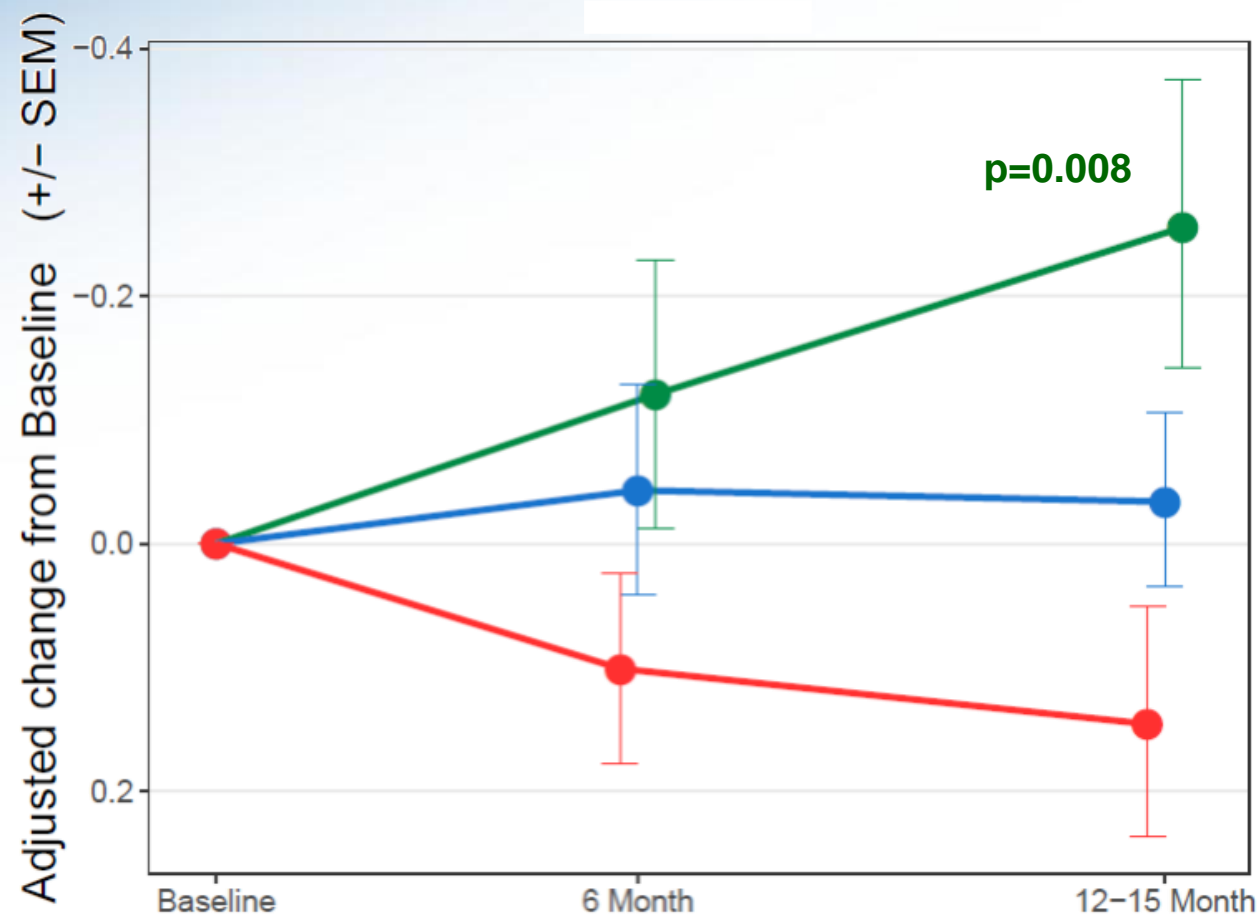
- **Protocole de l'étude PLEO-CMT**

- Pivot, randomisé, 15 mois, double aveugle, contrôlée vs placebo
- 30 centres au U.S., EU et Canada
- Formulation: solution liquide 2 fois par jour



ONLS : Overall Neuropathy Limitation Scale

# Essai de Phase 3 : PXT3003 améliore la maladie



Dose 2

Dose 1

Placebo

Dose 1: n=55 ; Dose 2: n=93 ; Dose 3: n=87

**PXT3003 a montré un très bon profil de sécurité**

# Prochaines étapes

**Q1 2019** : Réunion avec la FDA

**2H 2019** : Initiation de l'étude pédiatrique de Phase 3 du PXT3003 dans la CMT1A

**2H 2019** : Résultats de l'étude d'extension (PLEO-CMT-FU) sur la tolérance à long terme de PXT3003

**2H 2019** : Soumission des dossiers d'Autorisation de Mise sur le Marché à la FDA et l'EMA

**2020** : Lancement commercial

PXT864



# Nouveau mécanisme d'action : Restaure le déséquilibre chimique dans le cerveau

## Maladies en bref

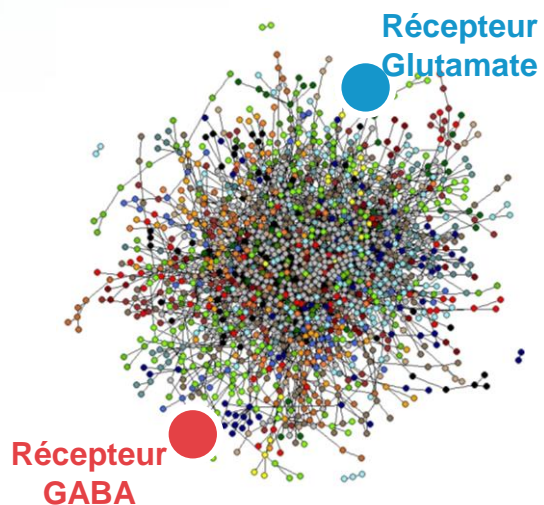
Alzheimer et autres maladies  
rares et communes

Cerveau Sain      Cerveau malade

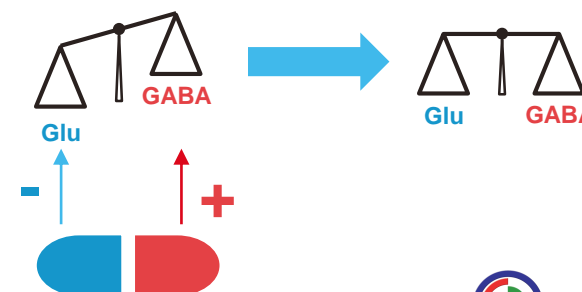
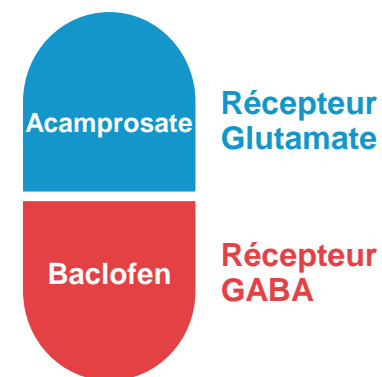


Glu: Glutamate

## malade Analyse du réseau

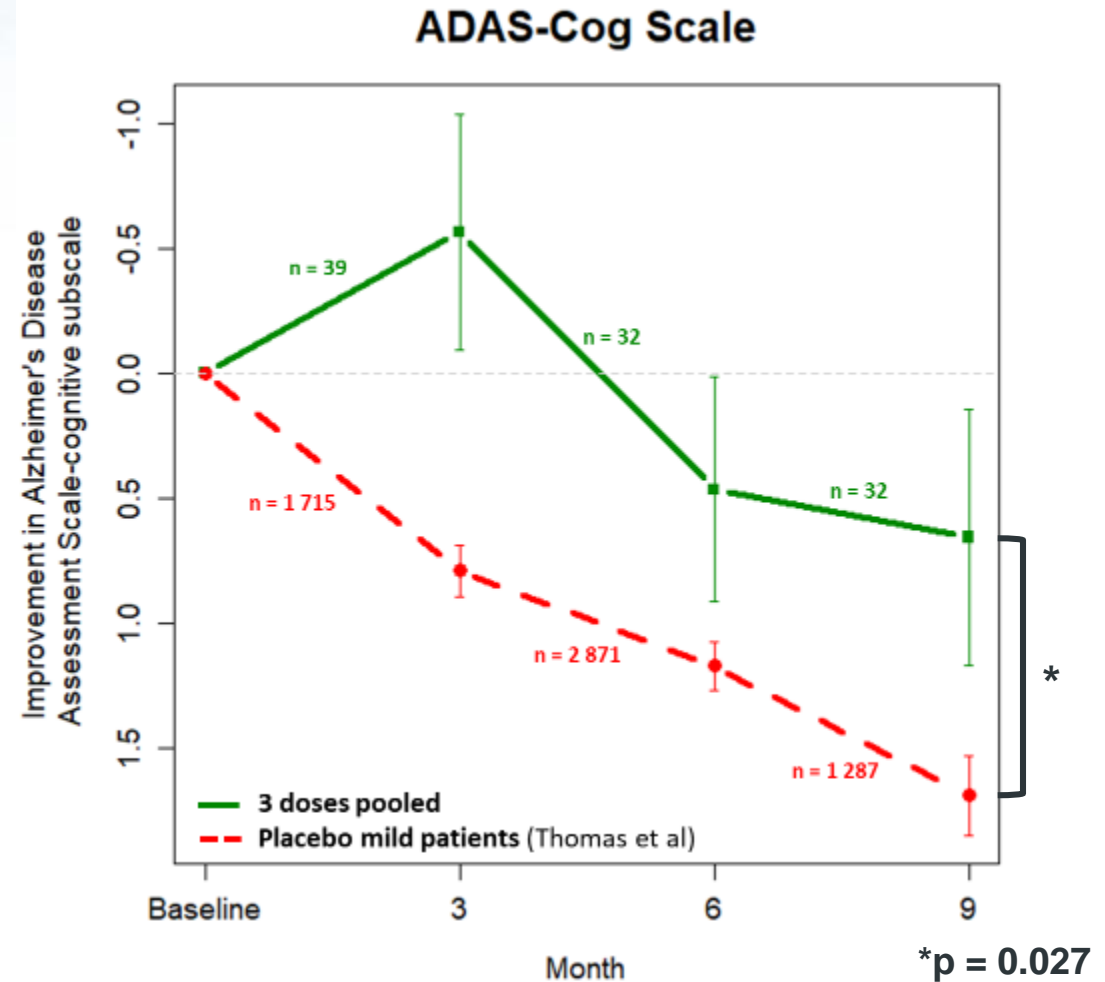


## PXT864



# Essai de Phase 2A de PXT864 dans Alzheimer : Amélioration et stabilisation

- Durée totale: 9 mois
- 45 Patients Alzheimer léger
- 12 critères cliniques d'efficacité
- 3 doses testées:
  - Baclofen: 12 – 30 mg (1/10)
  - Acamprosate: 1 – 40 mg (1/50)



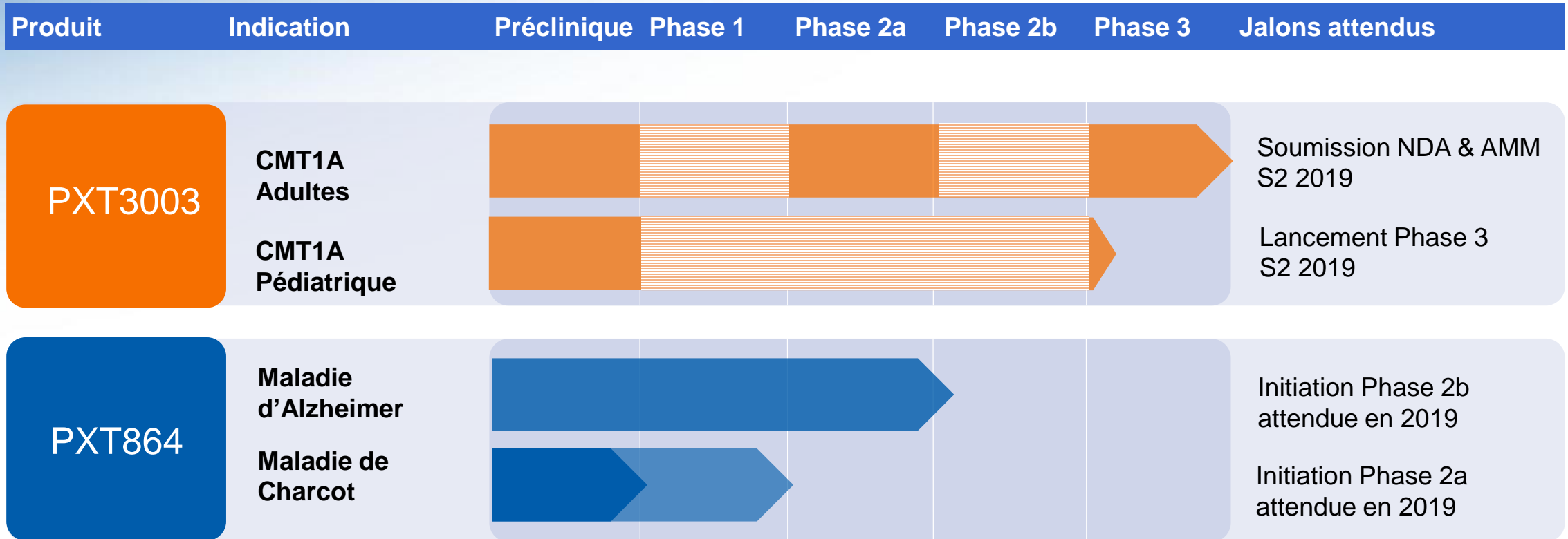
Abréviations : ADAS-Cog, Alzheimer's Disease Assessment Scale-cognitive.

PIPELINE





# Pipeline et Échéances Attendus



# Pipeline des Maladies

Le portefeuille cible des maladies rares ou communes protégées par une propriété intellectuelle robuste

## Orphanext Orphan Diseases

- Amyotrophic Lateral Sclerosis
- Acute idiopathic demyelinating polyneuropathy
- Autism subtypes
- Best vitelliform macular dystrophy
- Chronic inflammatory demyelinating polyneuropathy
- Charcot-Marie-Tooth type 2
- Cortical-basal ganglionic degeneration
- Creutzfeldt-Jacob disease
- Dysphasia
- Frontotemporal dementia
- Huntington's disease
- Multiple system atrophy
- Primary lateral sclerosis
- Progressive bulbar palsy
- Progressive supranuclear palsy
- Rett syndrome
- Spinal muscular atrophy
- Stargardt disease
- Tuberous sclerosis

## Partnerships Common Diseases

- Alzheimer's disease
- Parkinson's disease
- Age-related macular degeneration
- Alcohol addiction
- Alcohol withdrawal
- Alcoholic neuropathy
- Anosmia
- Anxiety
- Apraxia
- Bipolar disorder
- Chemo-induced peripheral neuropathies
- Diabetic neuropathies
- Diabetic retinopathies
- Dyspraxia
- Epilepsy
- Fibromyalgia
- Lewy body dementia
- Metabolic syndrome
- Mild cognitive impairment
- Multiple sclerosis
- Obesity
- Pain
- Parkinson's-dementia
- Peripheral nerve injury
- Schizophrenia
- Spinal cord injury
- Stroke
- Traumatic brain injury
- Type 1 diabetes
- Type 2 diabetes
- Vascular dementia
- Attention Deficit - hyperactivity disorder

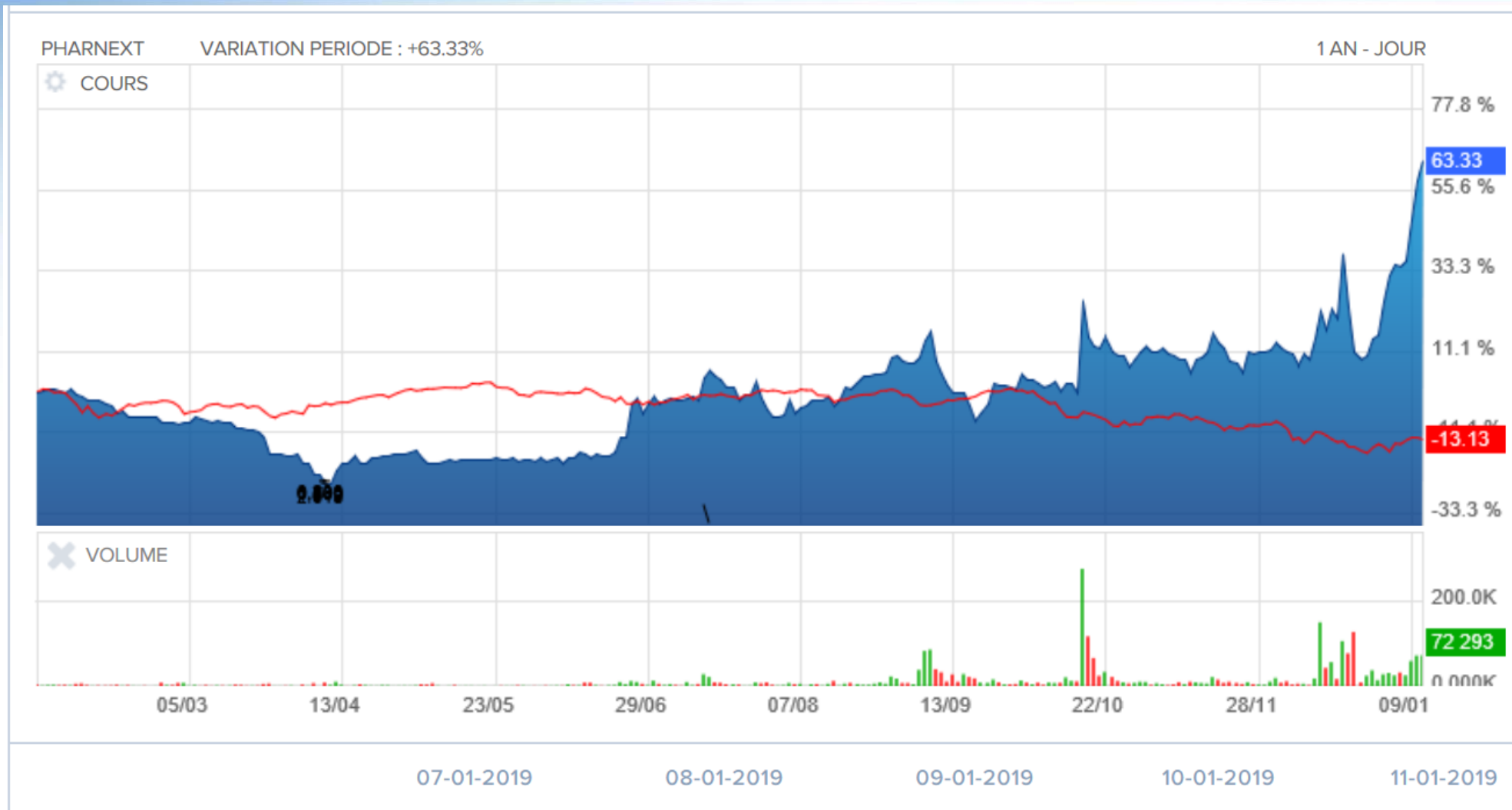
FINANCE



# Résultats au Premier semestre 2018

	Synthèse d'information financière (IFRS)	
En €K	30/06/2018	30/06/2017
Résultat opérationnel	2,430	
Autres revenus	2,376	1,216
Charges de Recherche et Développement	(8,925)	(7,610)
Charges Administratives	(3,289)	(2,936)
Résultat opérationnel	(7,407)	(9,331)
Résultat financier	(860)	(767)
Résultat net	(8,266)	(10,098)
Flux de trésorerie généré par les activités opérationnelles	(7,856)	(12,108)
Trésorerie liée aux activités d'investissement	13,602	1,740
Flux de trésorerie net	5,712	(10,521)
Trésorerie et équivalents de trésorerie au 30 juin	18,167	6,149
Trésorerie pro forma au 31 août 2018	30,959	

# Evolution du Prix de l'action Pharnext

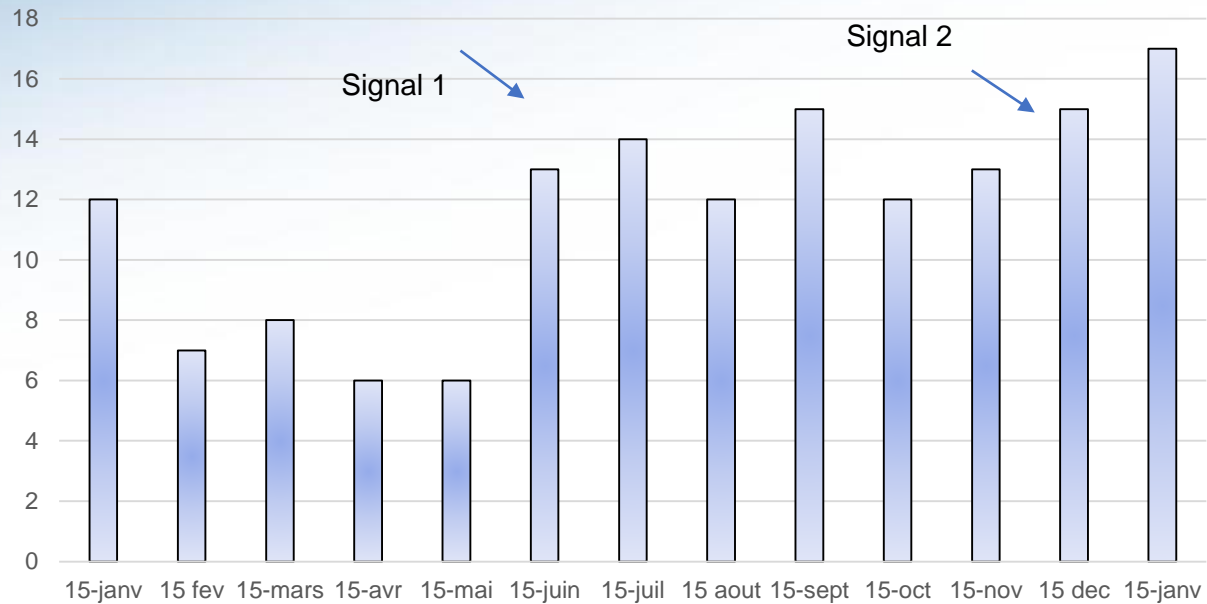


# Evolution du Prix de l'action Pharnext

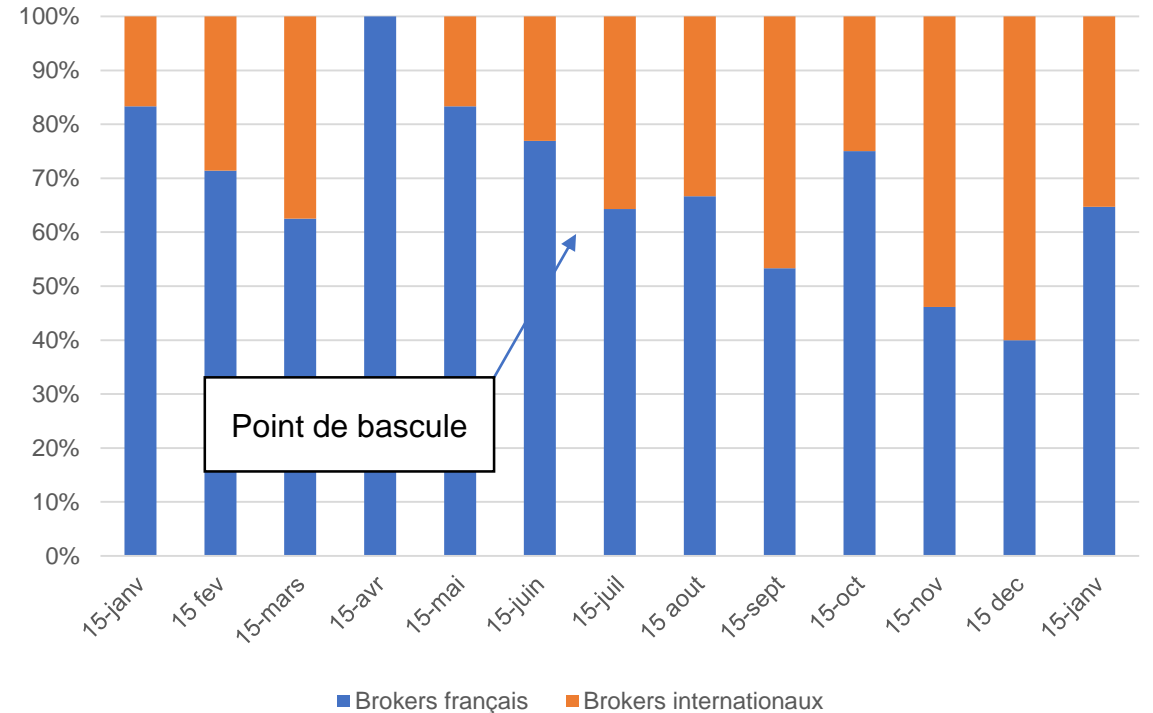


# Evolution des brokers sur le titre Alpha

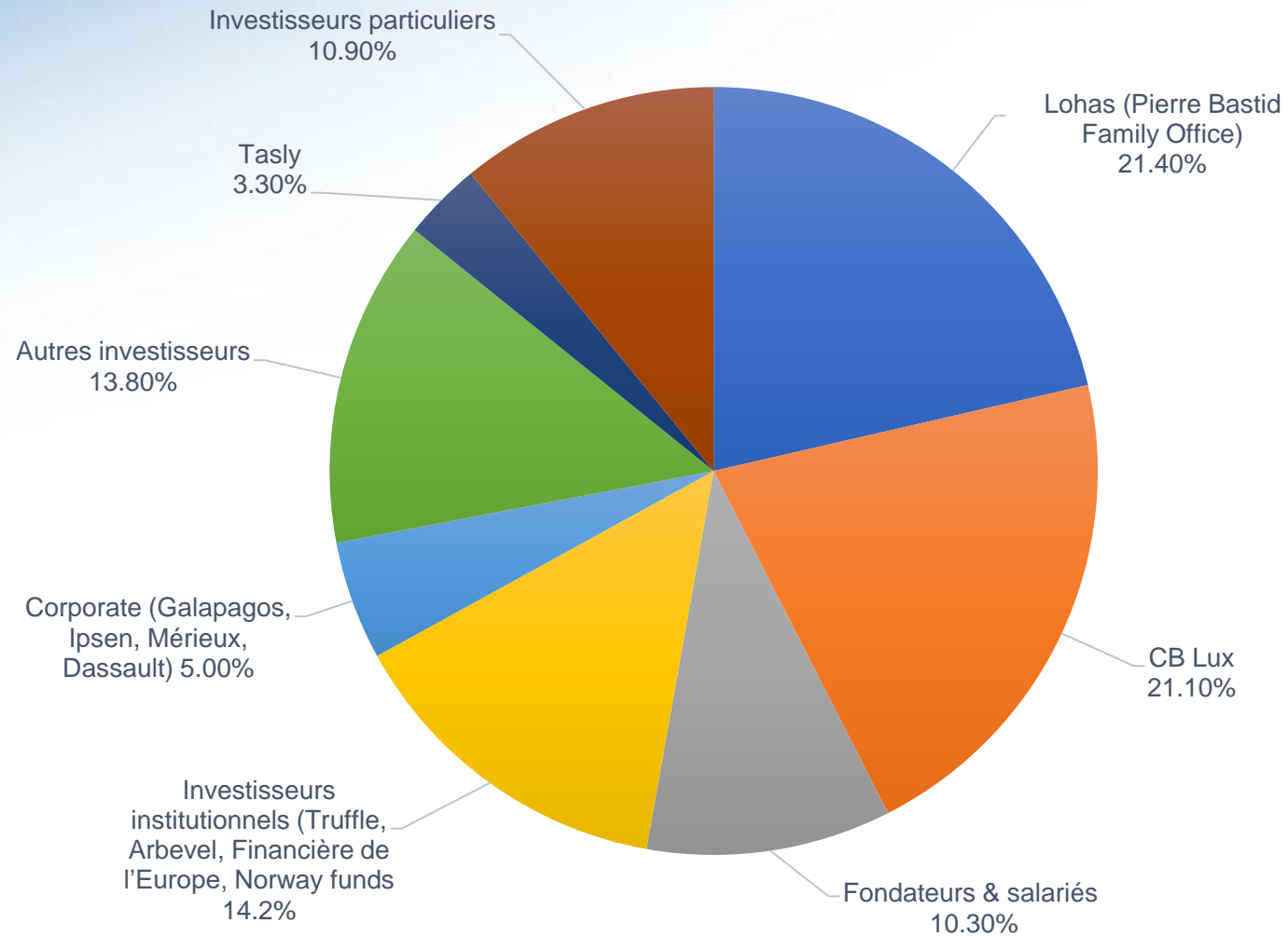
Nombre de brokers le 15 de chaque mois



Parts des brokers nationaux vs internationaux



# Répartition du capital





# Evolution du cours du titre Alpha



Gilbert Dupont (4 janvier 2019): 24,2€  
Kepler (13 décembre 2018): 20€

# Les évènements en 2019

- ❑ **Road shows à Paris avec Gilbert Dupont : le 15 janvier**
- ❑ **Agora Biotech à Paris : le 16 janvier**
- ❑ **BioMed à Paris: le 21 janvier**
- ❑ **Réunion Sfaf à Paris: le 12 février**
- ❑ **CMT Event : le 15 février**
- ❑ **Conférences prévues à Rennes, Lille et Lyon pendant le premier semestre 2019**

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