

PHARNEXT

16 janvier 2019, Hotel Bedford



Biotech
AGORA



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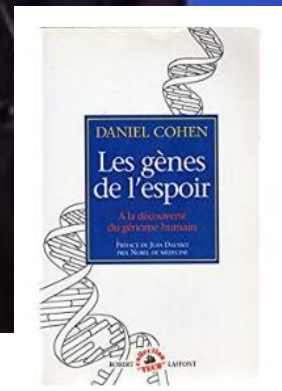
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Un fondateur pionnier

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

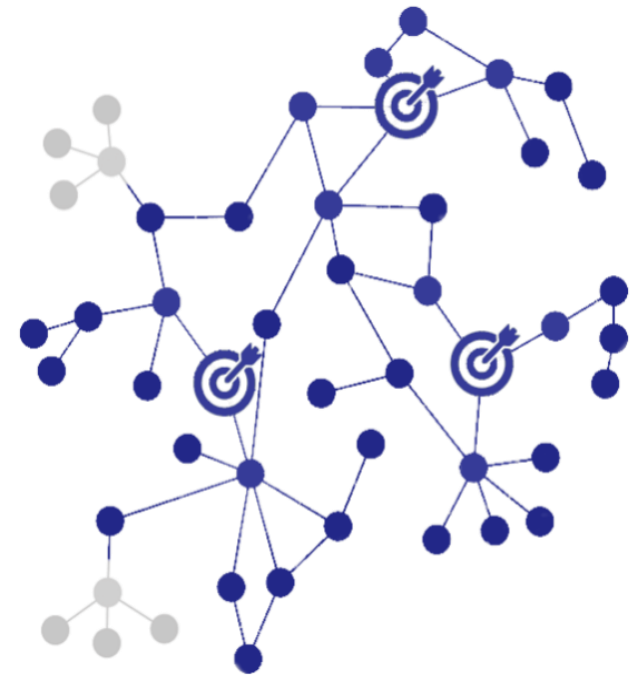
RESEAU DE LA MALADIE



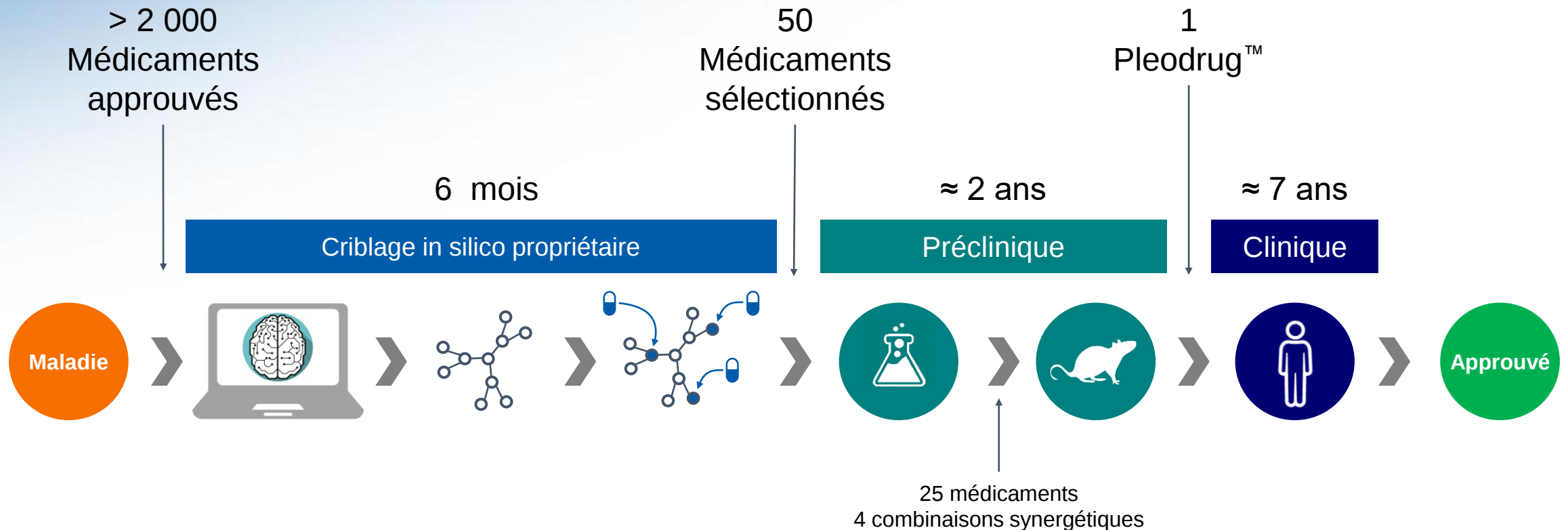
MONOTHERAPIE



PLEOTHERAPY™
(Approche de Pharnext)



Plateforme Universelle de R&D : Pleotherapy™



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

SYMPTOMES

Neuropathie héréditaire débilitante 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

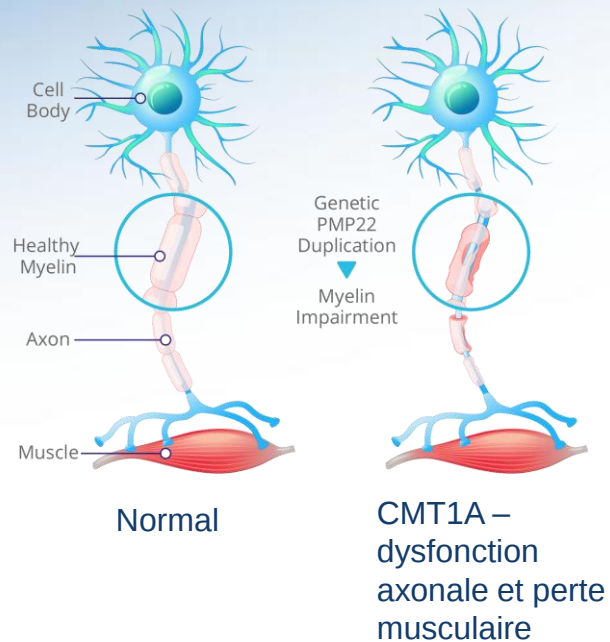
BARRIERES A L'ENTREE

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

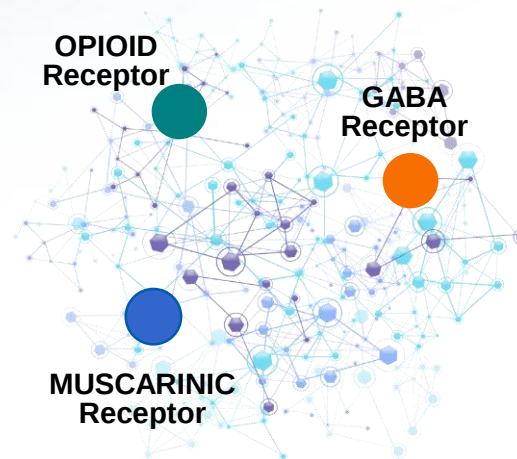


Conception et mécanisme du PXT3003

La maladie CMT1A en bref



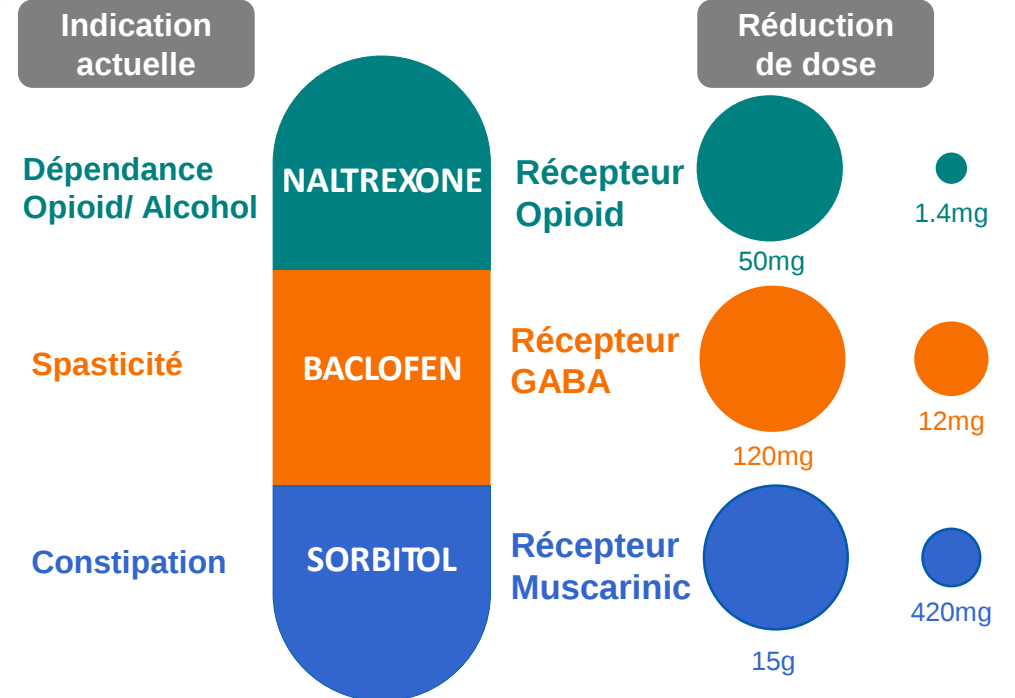
Analyse du réseau



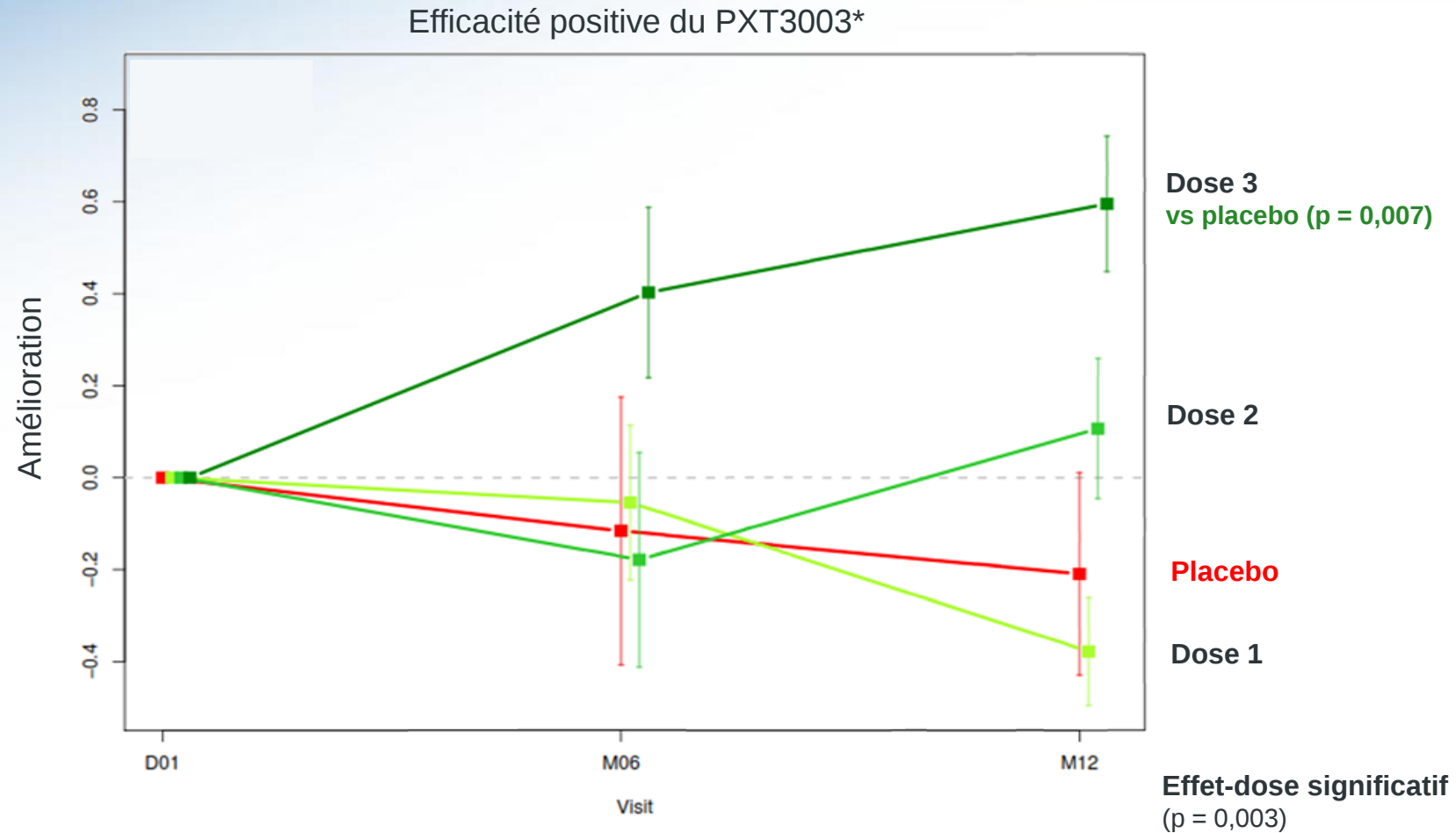
Amélioration de :

- 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.

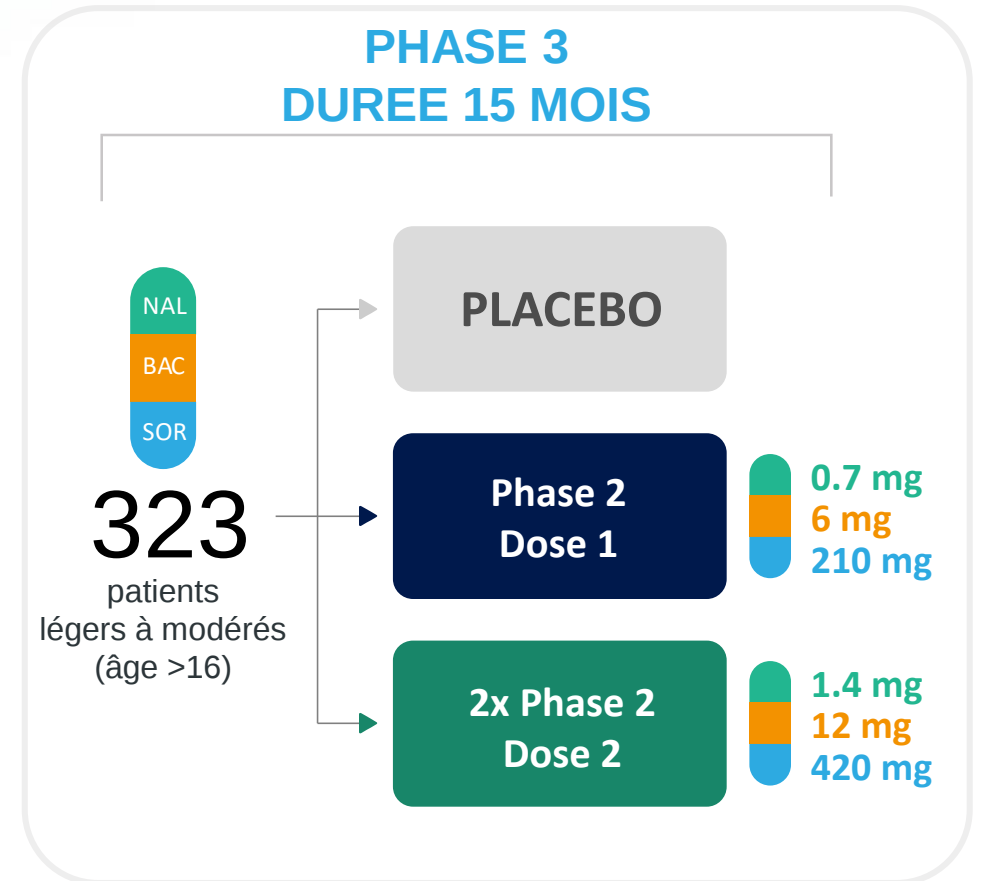
Essai de Phase 3 dans la CMT1A

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

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

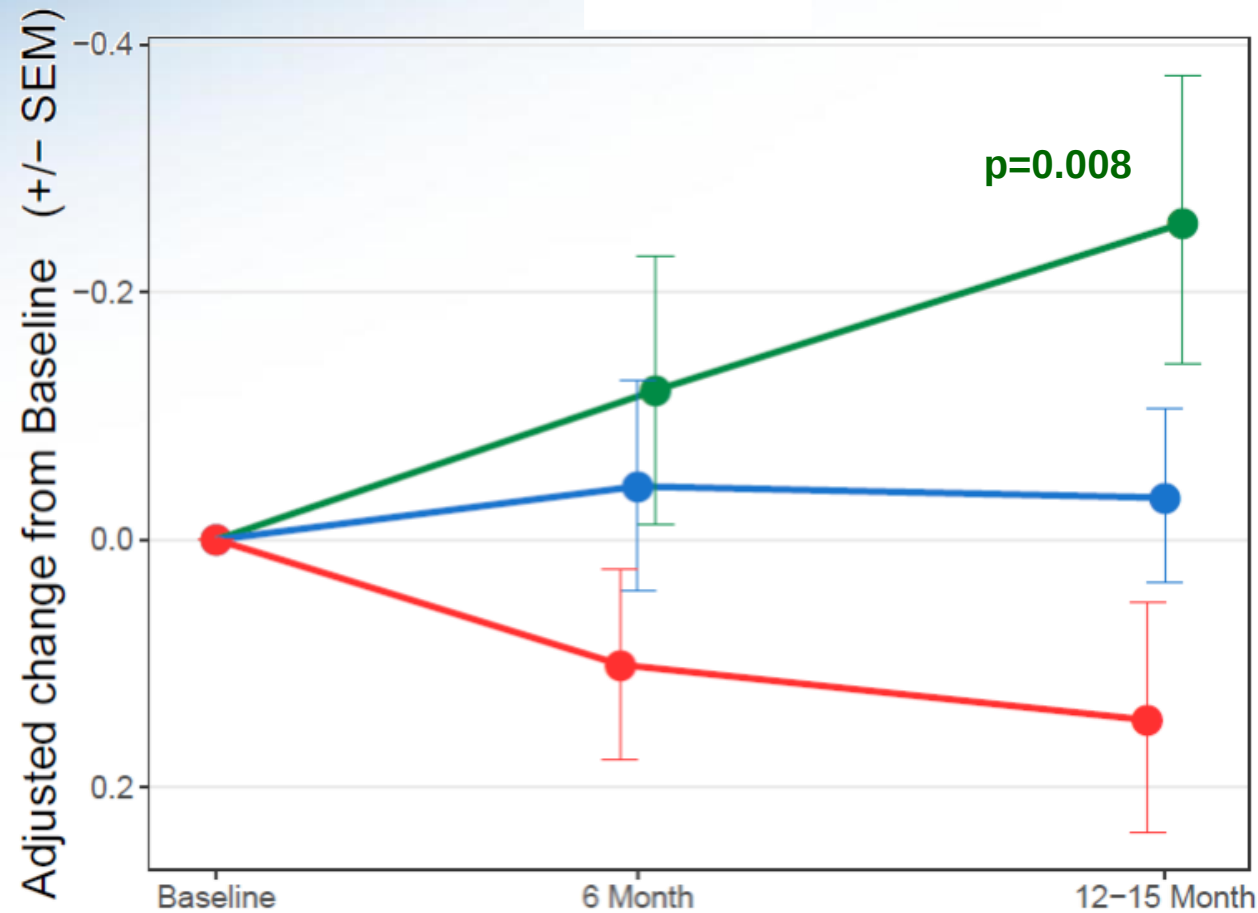
- **Protocole de l'étude PLEO-CMT**

- 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 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

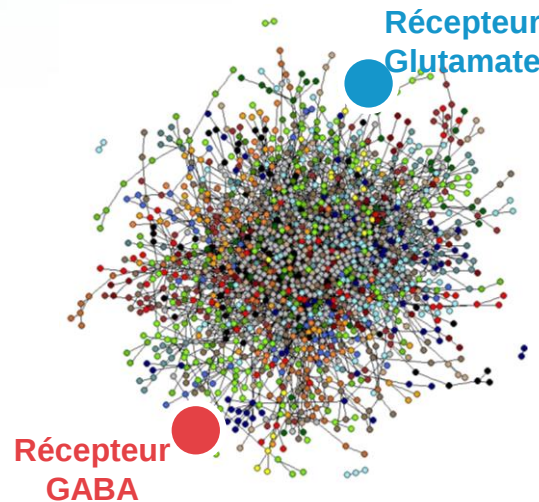


Cerveau malade

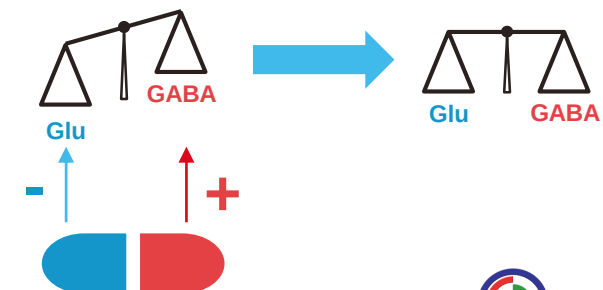
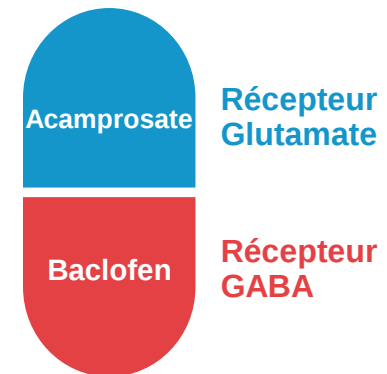


Glu: Glutamate

malade Analyse du réseau

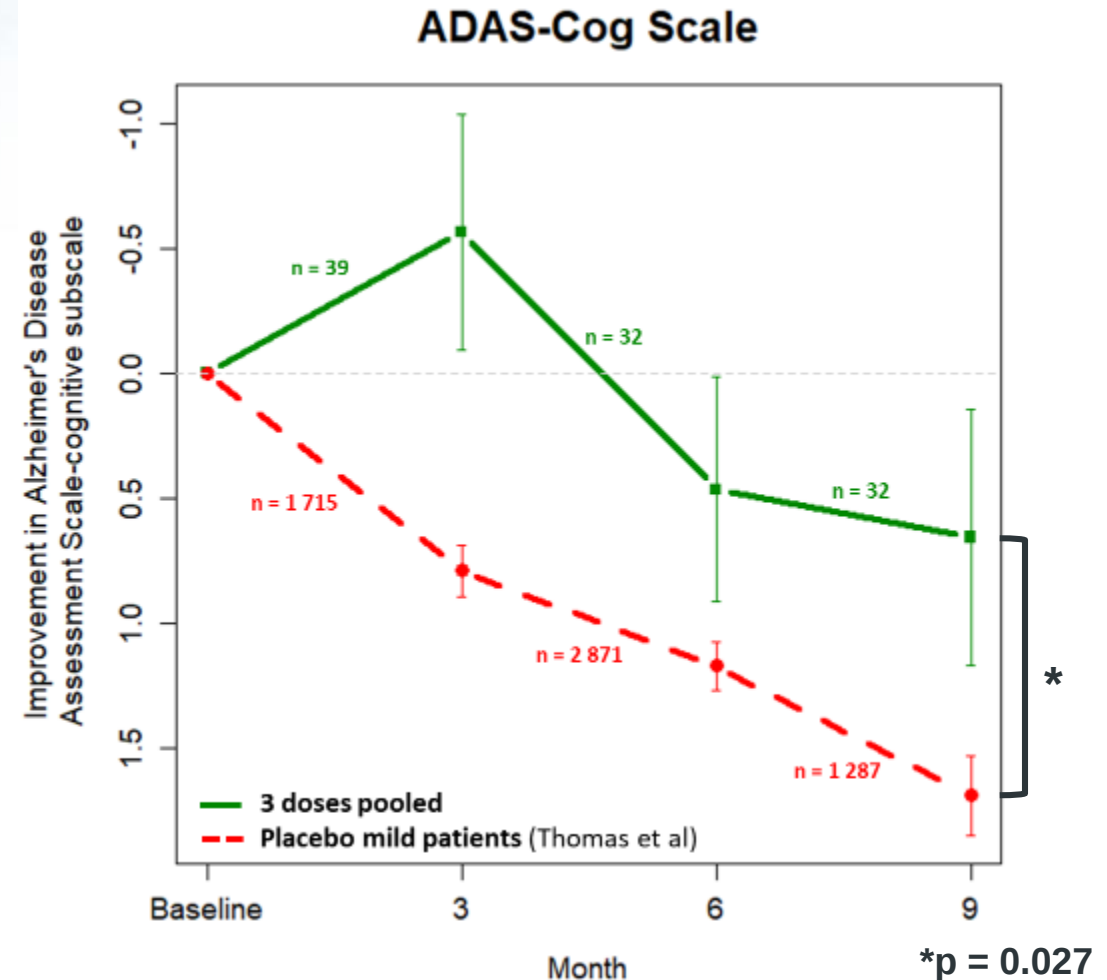


PXT864



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)

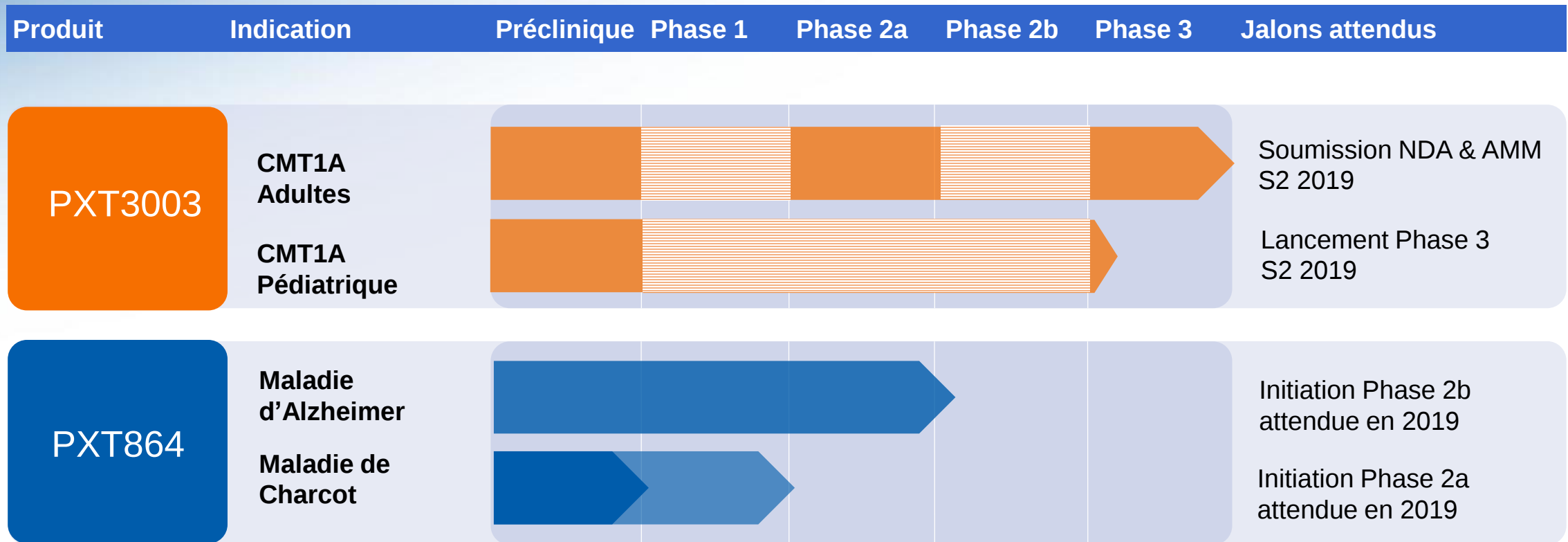


Abréviations : ADAS-Cog, Alzheimer's Disease Assessment Scale-cognitive.

PIPELINE



Pipeline et Échéances Attendus



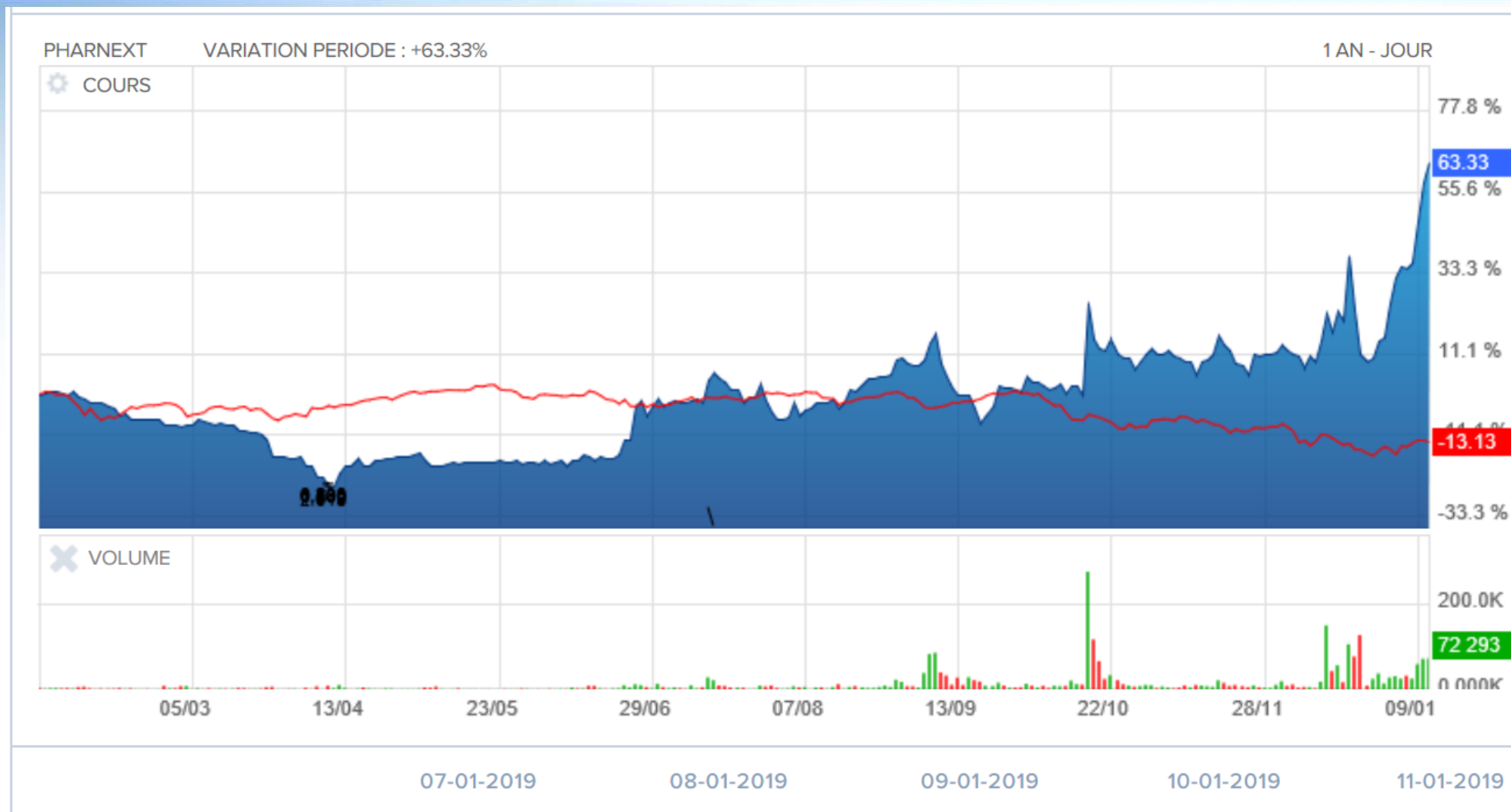
FINANCE



Résultats au Premier semestre 2018

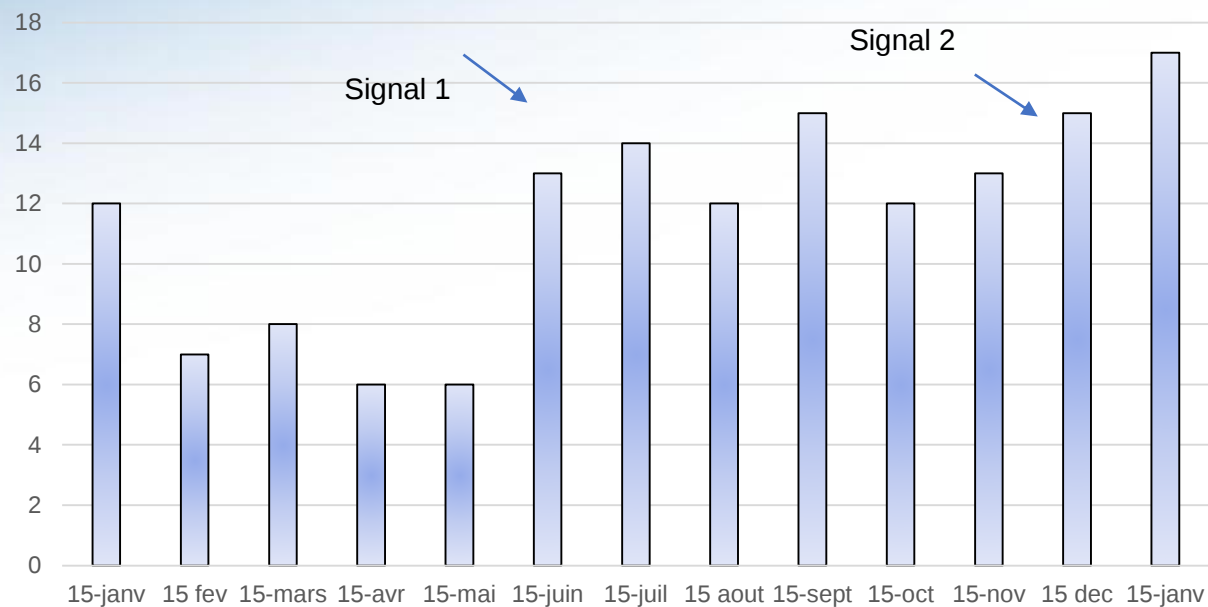
Synthèse d'information financière (IFRS)		
En €K	30/06/2018	30/06/2017
Résultat opérationnel	2,430	
Autres revenus	2,376	1,216
Charges de Recherche et Développement	(8,925)	(7,610)
Charges Administratives	(3,289)	(2,936)
Résultat opérationnel	(7,407)	(9,331)
Résultat financier	(860)	(767)
Résultat net	(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

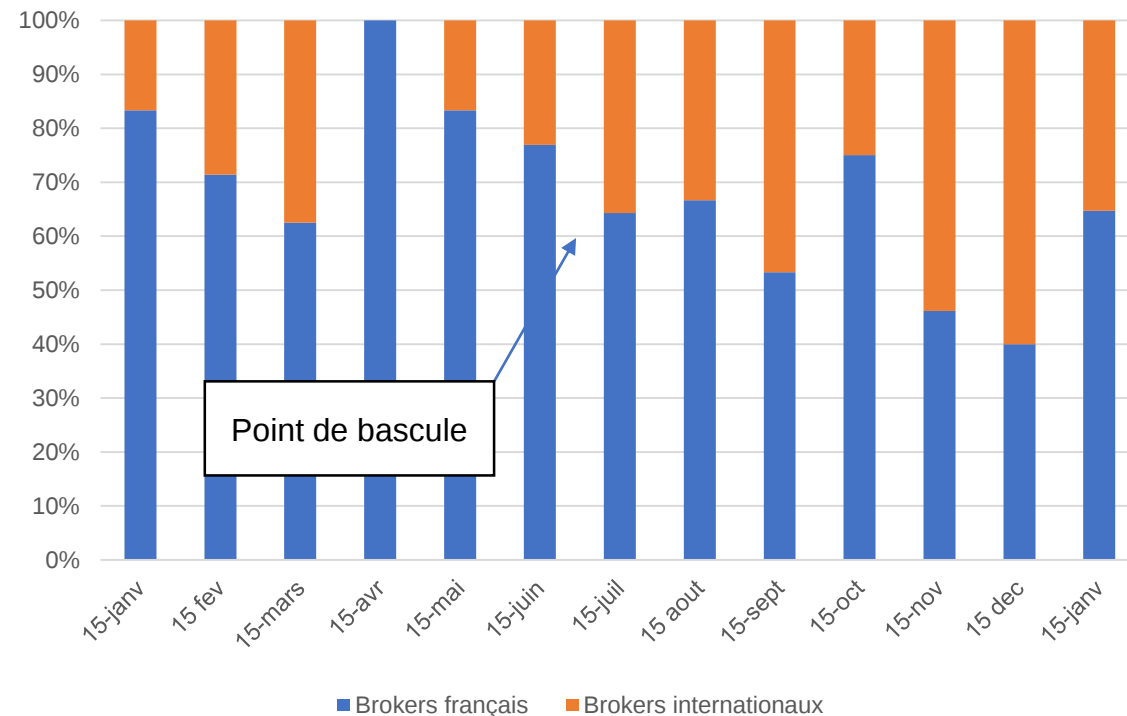


Evolution des brokers sur le titre Alpha

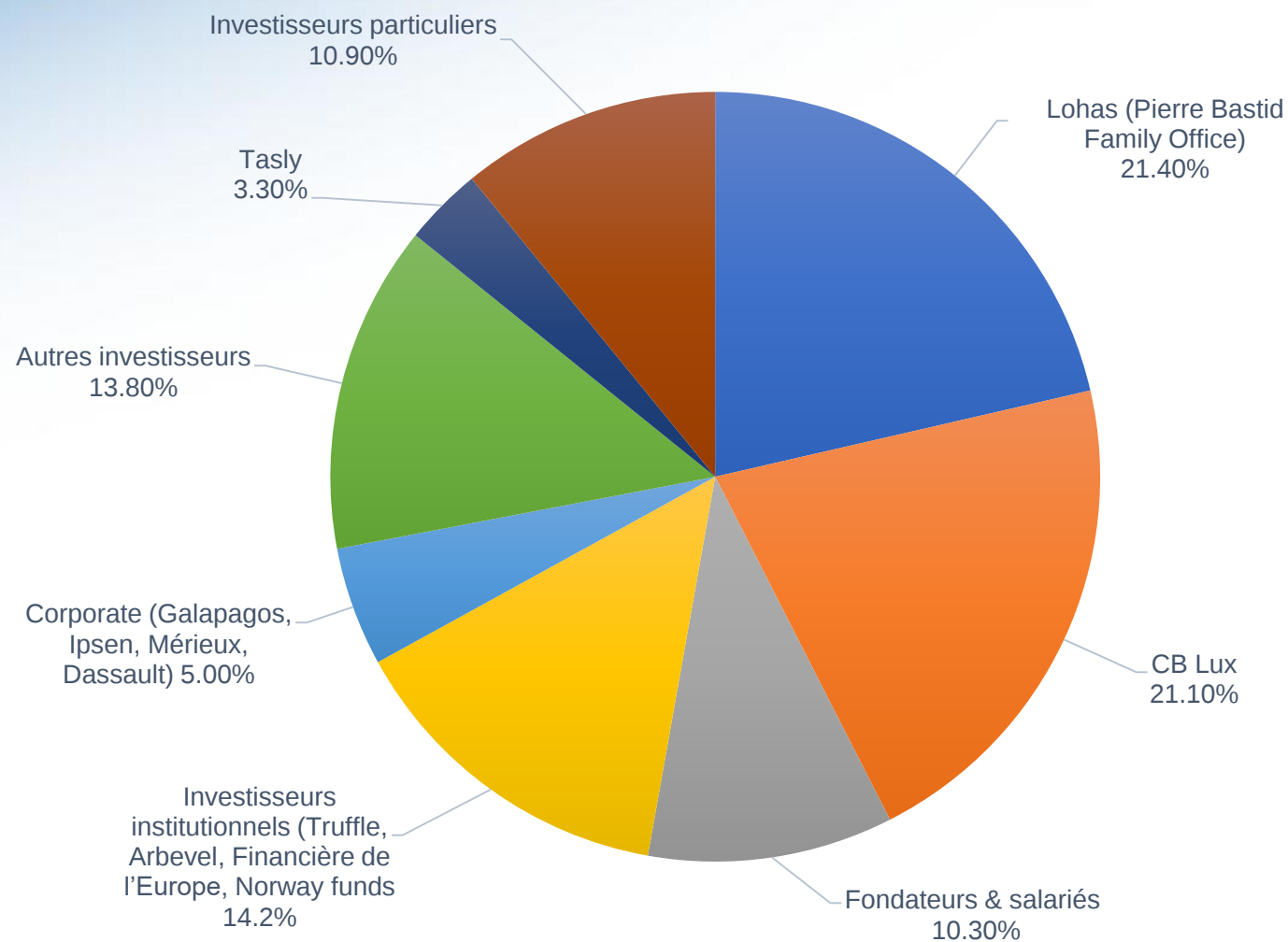
Nombre de brokers le 15 de chaque mois



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 22 janvier
- ❑ Réunion Sfaf à Paris: le 12 février
- ❑ CMT Event à Paris : le 1^{er} mars
- ❑ Conférences prévues à Rennes, Lille et Lyon pendant le premier semestre 2019

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