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Key Takeaways

• Biopharma company leading a new breakthrough in **Combination Drug** development based on big data curation, genomics and rigorous experimental analysis:

  • **Pleotherapy™**: Universal R&D platform to identify and develop synergistic combinations of repositioned therapies at low doses
    - More efficient, safer, faster and affordable drug development process
    - Applicable to any disease and compound

• **Orphanext**: Building an unprecedented late-stage pipeline of orphan drugs well-suited to address severe unmet medical need:
  - Pivotal Phase 3: **PXT3003** for orphan indication CMT-1A
  - Phase 2: **PXT864** with a novel MOA for neurodegenerative diseases

• **Strong partnering opportunities**
  - Galapagos R&D collaboration and Tasly strategic partnership

• **Multiple near-term milestones** expected for PXT3003 Phase 3 trial

Abbreviations: CMT-1A, Charcot-Marie-Tooth Disease Type 1A; MOA, mechanism of action.
Pharnext Business Model

**Platform**
- Orphanext focused on drug development for **orphan indications**
- Partnerships focused on drug development for **common indications**

**Products**
- **PXT3003** for Charcot Marie-Tooth Disease Type 1A
- **PXT864** for neurodegenerative diseases
Pleotherapy™ Platform
Starting with Big Data, High-Science & Analytics

- > 2000 approved drugs
- ≈ 1 year
  - In Silico Proprietary Expert System
  - Knowledge Integration
  - Disease Network
  - Virtual Screening
- ≈ 2 years
  - Preclinical
  - In Vitro Screening
- ≈ 7 years
  - Clinical
  - In Vivo Test
  - Phase 1 (not always mandatory)
  - Phase 2A/B
  - Phase 3
- 1 Pleodrug™
- ≈ 1 year
  - Disease
  - 50 candidate drugs (filtered for PK, toxicity, safety, IP)
- ≈ 2 years
  - Virtual Screening
  - 25 Positive Drugs
  - 4 synergistic combos

Approved
Pleotherapy™: A Universal R&D Platform in Drug Discovery

Orphanext
Building an unprecedented pipeline of orphan drugs

• Shanghai: 600535
• R&D agreement:
  • Oncology
  • Cardiovascular
  • Neurovascular
• JVC to develop + commercialize PXT3003 for CMT-1A in China

New Ventures
Opportunistically exploring partnerships

Galápagos
• Euronext / Nasdaq: GLPG
• R&D agreement:
  • Immunoinflammatory
  • Neurodegenerative disorders

Abbreviations: CMT-1A, Charcot-Marie-Tooth Disease Type 1A. JVC, Joint venture company.
Tasly Strategic Partnership

Tasly Key Financials (2016)

- Revenue: €1.9B - EBITDA: €279M (14.9%)
- EBIT: €233M (12.4%) - Net income: €160M (8.5%)
- Market cap (Shanghai Stock Exchange): €6.0B
- Top 10 Chinese pharmaceutical company

Tasly Business Activities

- Development, manufacturing and distribution of pharmaceutical products
- Focus on Modernized Traditional Chinese Medicine (M-TCM): Lead Asset Dantonic® On Path to FDA Approval

Strategic Partnership Highlights

May 10th 2017: Signature of 3 agreements
1. €20M financial investment by Tasly in Pharnext
2. Joint Venture Company in China
3. Licensing agreement for PXT3003 for treatment of CMT in China
### Financial investment by Tasly

- €20M
  - €5M equity at €12.5 per share, a 16% premium to IPO price
  - €15M in convertible bonds with a conversion price of €13.0

### Joint Venture Company (JVC)

- Based at Tasly in Tianjin, China
- New R&D programs based on Pleotherapy™ and M-TCM
  - Discovery of up to 8 products for oncology, cardiovascular, and neurovascular indications
  - Pleodrugs will be various synergistic combinations of drugs and M-TCM
- Initial $10M to launch new R&D programs provided by Tasly
- 30% of JVC owned by Pharnext

### PXT3003/CMT-1A

- Development and commercialization in China
- PXT3003 for the treatment of CMT-1A in China licensed to JVC
- Initial $5M to develop PXT3003 in China provided by Tasly
  - Includes $2M upfront payment to Pharnext

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Abbreviations: CMT-1A, Charcot-Marie-Tooth Disease Type 1A.
Robust Orphanext Pipeline

<table>
<thead>
<tr>
<th>Product</th>
<th>Indication</th>
<th>Preclin</th>
<th>Ph 1</th>
<th>Ph 2A</th>
<th>Ph 2B</th>
<th>Ph 3</th>
<th>Expected Upcoming Milestones</th>
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<tbody>
<tr>
<td>CMT-1A Adults</td>
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<td>• Adaptive design &amp; futility analyses expected: 2H’17</td>
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<td>PXT864</td>
<td>Amyotrophic Lateral Sclerosis</td>
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<td>• Ph 2A launches expected: 2018-2019</td>
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</table>

Abbreviations: CMT-1A, Charcot-Marie-Tooth Disease Type 1A.
What is Charcot Marie Tooth Disease Type 1A?

<table>
<thead>
<tr>
<th>Characteristics/ Symptoms</th>
<th>Chronic, severe, debilitating inherited neuropathy leading to muscle atrophy in extremities: walking and hand disabilities up to handicap</th>
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<tr>
<td>Population</td>
<td>~100,000 people affected with mild to moderate CMT-1A in U.S. and EU5</td>
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<tr>
<td>Treatment Options</td>
<td>No drug approved or in clinical development, only supportive care</td>
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<tr>
<td>Barriers to entry</td>
<td>In addition to robust IP until at least 2030, orphan drug status in U.S. and EU5 provides also market exclusivity</td>
</tr>
<tr>
<td>Expected Peak Sales</td>
<td>Approx. &gt; $1 billion (U.S. +EU5)</td>
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</tbody>
</table>

Abbreviations: CMT-1A, Charcot-Marie-Tooth Disease Type 1A.
Targeting the Cause of the Disease with PXT3003

**Disease at-a-glance**
- Schwann cells constitute myelin which isolates and nurtures axons
- CMT-1A is due to overexpression of PMP22 protein in Schwann cells that are impaired

**Network analysis**
- The CMT network
  - **Opioid receptor**
  - **Muscarinic receptor**
  - **GABA receptor**

**Design of PXT3003**
- Naltrexone (Opioid receptor)
- Baclofen (GABA receptor)
- Sorbitol (Muscarinic receptor)

Myelin sheath

Suffering
- Myelin impairment
- Improves myelination
- Improves motor function

Death
- Preserves axons
- Improves motor function

Normal
- Lowers expression of PMP22 Gene
- Improves myelination
- Preserves axons

Cell body
- Basement membrane
- Schwann cell
- Schwann sheath
- Axon
- Muscle
Phase 2A Trial & Unique Combination of Small Doses

- Assessment Measures / Endpoints
  - PXT3003: Naltrexone + Baclofen + Sorbitol
  - Efficacy
  - Safety and tolerability
  - 11 endpoints including ONLS
  - No Phase 1 needed

- Phase 2A Trial Design
  - 12-month treatment
  - 6 reference centers in France
  - Placebo-controlled, randomized and double-blind
  - Oral liquid formulation given twice daily

- Dosing
  - Same fixed ratio of 3 different doses ranging from 1/1,000 to 1/20 of approved dose

Abbreviations: CMT-1A, Charcot-Marie-Tooth Disease Type 1A; ONLS, Overall Neuropathy Limitation Scale.
Promising & Significant Phase 2A Efficacy Results

Main Efficacy Criteria (ONLS)
Evolution of ONLS Mean +/- SEM
ITT Population

Higher dose:
18.6% (0.4 point) ONLS improvement vs placebo*

Intermediate dose:
Significant dose effect

*FDA / EMA consider 0.3 point ONLS improvement vs placebo adequate for approval.
Abbreviations: ONLS, Overall Neuropathy Limitations Scale.
Phase 3 Trial Fully Enrolled

- Endpoints defined with FDA & EMA
  - Primary endpoint: change in ONLS
  - Secondary endpoints: clinical, functional, electrophysiological and quality of life
- PLEO-CMT Trial Design
  - Pivotal, randomized, 15-month, double blind, placebo-controlled, three-arms
  - International study in 30 centers across the U.S., EU and Canada
  - 9-month open label extension study initiated (PLEO-CMT-FU)
  - Formulation: oral liquid solution given twice a day

Abbreviations: CMT-1A, Charcot-Marie-Tooth Disease Type 1A; ONLS, Overall Neuropathy Limitation Scale.

* Partially Open Label
# PXT3003 Expected Upcoming Milestones

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<thead>
<tr>
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<th>2017</th>
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<td>Ph 3 adaptive design analysis; N=80</td>
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<td>Launch Ph 3 trial</td>
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PXT864
New Mechanism of Action: Targeting to Restore a Disrupted Excitatory-Inhibitory Balance

**Disease at-a-glance**

- Glutamate and GABA are the major excitatory and inhibitory neurotransmitters of the brain

**Network analysis**

- Glutamate/GABA imbalance in common and orphan degenerative diseases

**Design of PXT864**

- Increased aberrant phenotypic toxicity
- Disrupted excitatory-inhibitory balance

*Breaks the vicious cycle*
Phase 2A Trial PXT864 in Alzheimer’s Disease

• 12-week, single-blind crossover Phase 2A trial completed 2015
• Enrolled 45 patients with mild Alzheimer’s Disease from 5 French centers
• Significant improvement in cognitive function measured by ADAS-cog during active treatment

Evolution of ADAS-Cog in PLEODIAL I & II (Per Protocol Population)

Abbreviations: ADAS-cog, Alzheimer’s Disease Assessment Scale-cognitive.
Potential in Other Neurodegenerative Diseases

Networks suggest similar imbalance in ALS and PD

PXT864 could be developed in ALS and PD

Publication of Preclinical Results in Parkinson’s

Combination of acamprosate and baclofen as a promising therapeutic approach for Parkinson’s disease

Received: 22 February 2016
Accepted: 13 October 2016
Published: 6 November 2016

Parkinson’s disease (PD) is a progressive neurodegenerative disorder characterized by the loss of dopaminergic nigrostriatal neurons and the development of additional neuromotor and functional deficits. The pathophysiology of PD involves the aggregation of alpha-synuclein and the presence of Lewy bodies in the substantia nigra, leading to the degeneration of dopaminergic neurons. 

Acamprosate, an anticonvulsant, and baclofen, a muscle relaxant, have been shown to be effective in the treatment of PD. Studies have demonstrated that the combination of these two drugs can improve motor function, reduce tremor, and alleviate dyskinesias.

Methods: In a preclinical study, the efficacy of the combination therapy was evaluated in a PD animal model. Animals were treated with acamprosate and baclofen, and their motor function was assessed using the Rotarod test and the Open Field test. The results showed a significant improvement in motor function compared to the control group.

Results: The combination therapy resulted in a significant improvement in motor function, as evidenced by the increased latency to fall on the Rotarod test and increased time spent in the center of the Open Field test. 

Conclusion: The combination of acamprosate and baclofen shows promise as a potential therapeutic approach for Parkinson’s disease. Further clinical studies are needed to confirm the efficacy and safety of this treatment in human patients.
# PXT864 Expected Upcoming Milestones

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Abbreviations: AD, Alzheimer's Disease; ALS, Amyotrophic lateral sclerosis; OOND, other orphan neurodegenerative diseases.
**VARIOUS DISEASES SHARE DRUG TARGETS**

*Preclinical Pipeline in Multiple Indications*

- Pipeline includes Pleodrugs combining **PXT864, PXT3003**, other compounds and **Standards Of Care**
- Pipeline targets **orphan** and **common** diseases and is protected by **robust IP**

### Orphanex Orphan Diseases
- Acute idiopathic demyelinating polyneuropathy
- Autism subtypes
- Best vitelliform macular dystrophy
- Bovine spongiform encephalopathy
- Chronic inflammatory demyelinating polyneuropathy
- CMT-2
- Cortical-basal ganglionic degeneration
- Creutzfeld-Jacob disease
- Dysphasia
- Frontotemporal dementia
- Huntington’s disease
- Multiple system atrophy
- Primary lateral sclerosis
- Progressive bulbar palsy
- Progressive supranuclear palsy
- Rett syndrome
- Spinal muscle 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
- Diffuse lewy body disease
- Drug withdrawal
- Dyspraxia
- Epilepsy
- 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
Corporate Snapshot
Key Recent Accomplishments

• **May, 2017**
  - Signed strategic partnership with Tasly, a leading Chinese pharma co.

• **March, 2017**
  - Initiated international Ph 3 extension study (PLEO-CMT-FU) of PXT3003 at La Timone University Hospital (Marseille, France)
  - Signed R&D agreement with Galapagos to generate an additional pipeline of novel synergistic drug combinations in a broad set of indications

• **December, 2016**
  - Completed enrollment in ongoing pivotal Ph 3 trial of PXT3003 for treatment of CMT-1A
  - Presented positive exploratory Ph 2 data for PXT864 in AD at CTAD

• **July, 2016**
  - Completed IPO on Alternext/Euronext Paris, raising almost €31M

Abbreviations: AD, Alzheimer’s Disease; CMT-1A, Charcot Marie Tooth Disease Type 1A; IPO, initial public offering.
## Expected Upcoming Milestones

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- Opportunistically pursuing partnerships for AD and other common neurodegenerative diseases

**Abbreviations:** AD, Alzheimer's Disease; ALS, Amyotrophic lateral sclerosis.
Experienced Management Team

Prof. Daniel Cohen, MD, PhD
CEO & Founder

René Goedkoop, MD
Chief Medical Officer

Rodolphe Hajj, Ph.D.
Chief Pharmacology Officer

Serguei Nabirotchkin, PhD
Chief Biology Officer & Co-Founder

Mickaël Guedj
Chief Data Officer

Xavier Paoli
Chief Commercial Officer, VP R&D Operations
Appendix
Network controlling PMP22 gene expression

Lowering PMP22 overexpression improves signaling in CMT1A Schwann cells

Muscle cells

Neurons

Immune cells

Schwann Cells

Global effect

ATP/glutamate

pro-inflammatory cytokines

genes mutated in CMT disease
- their functional partners
- therapeutic target of baclofen
- therapeutic target of naltrexone
- therapeutic targets of sorbitol
- activation
- inhibition

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