PLEO-CMT Top-line Results

Presentation

October 16, 2018

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PLEO-CMT Pivotal Phase 3 Topline Results

- **PLEO-CMT:** International, multi-center, randomized, double-blind, placebo-controlled pivotal Phase 3 study
- **Primary endpoint:** Mean reduction in disability measured by the change in Overall Neuropathy Limitation Scale (ONLS) compared to placebo at 12 and 15 months of treatment
- Summary of results:
  - **Higher dose met primary endpoint, the Overall Neuropathy Limitation Scale (ONLS), with statistical significance (p = 0.008)**
  - **Higher dose met secondary endpoint, the 10-meter walk test, with statistical significance (p=0.016)**
  - **Safe and well tolerated, no treatment related SAE in the higher dose group**
  - **Based on these positive results, Pharnext intends to file for market approval in the US and Europe**

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Charcot-Marie-Tooth Disease Type 1A

SYMPTOMS
Chronic, severe, debilitating inherited neuropathy leading to muscle atrophy in extremities: walking and hand disabilities up to handicap

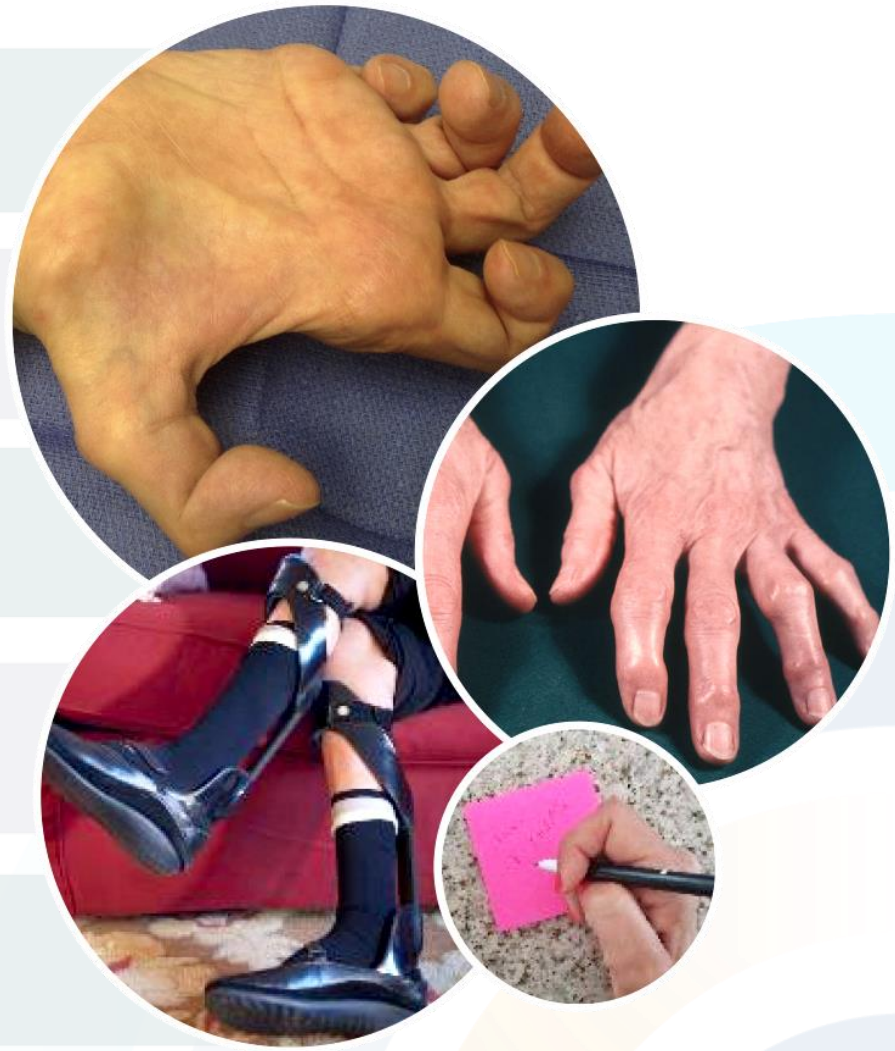
DIAGNOSIS
About 50% of patients have symptoms before the age of 20 confirmed by genetic testing

POPULATION
~100,000 people affected with mild to moderate CMT1A in US and EU5

TREATMENT OPTIONS
No drug approved

Only supportive care available

BARRIERS TO ENTRY
In addition to robust IP until at least 2030, orphan drug status in US and EU5 provides market exclusivity for 7 years and 10 years, respectively



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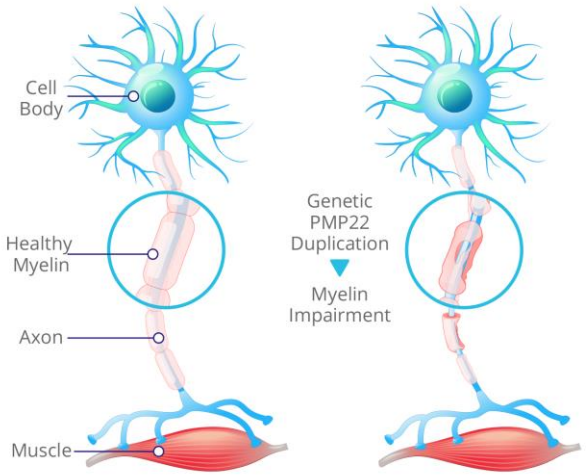
PXT3003 Design and Mechanism of Action

Preclinical data demonstrate that PXT3003 acts on different cell types of the motor unit in CMT1A by:

- improving the known dysbalance of intracellular signalling pathways (Akt/Erk) in Schwann cells by downregulation of PMP22 expression
- increasing the axonal diameter and the number of myelinated axons in peripheral nerve
- restoring the number of functional neuromuscular junctions and fast-contracting muscle fibers

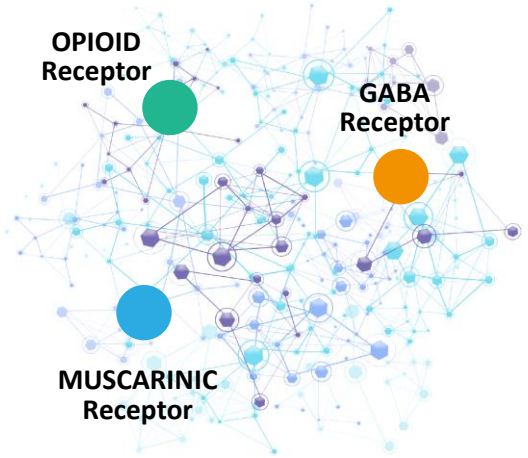
CMT1A disease at-a-glance



Normal

CMT1A - Axonal dysfunction and muscle loss

Network analysis

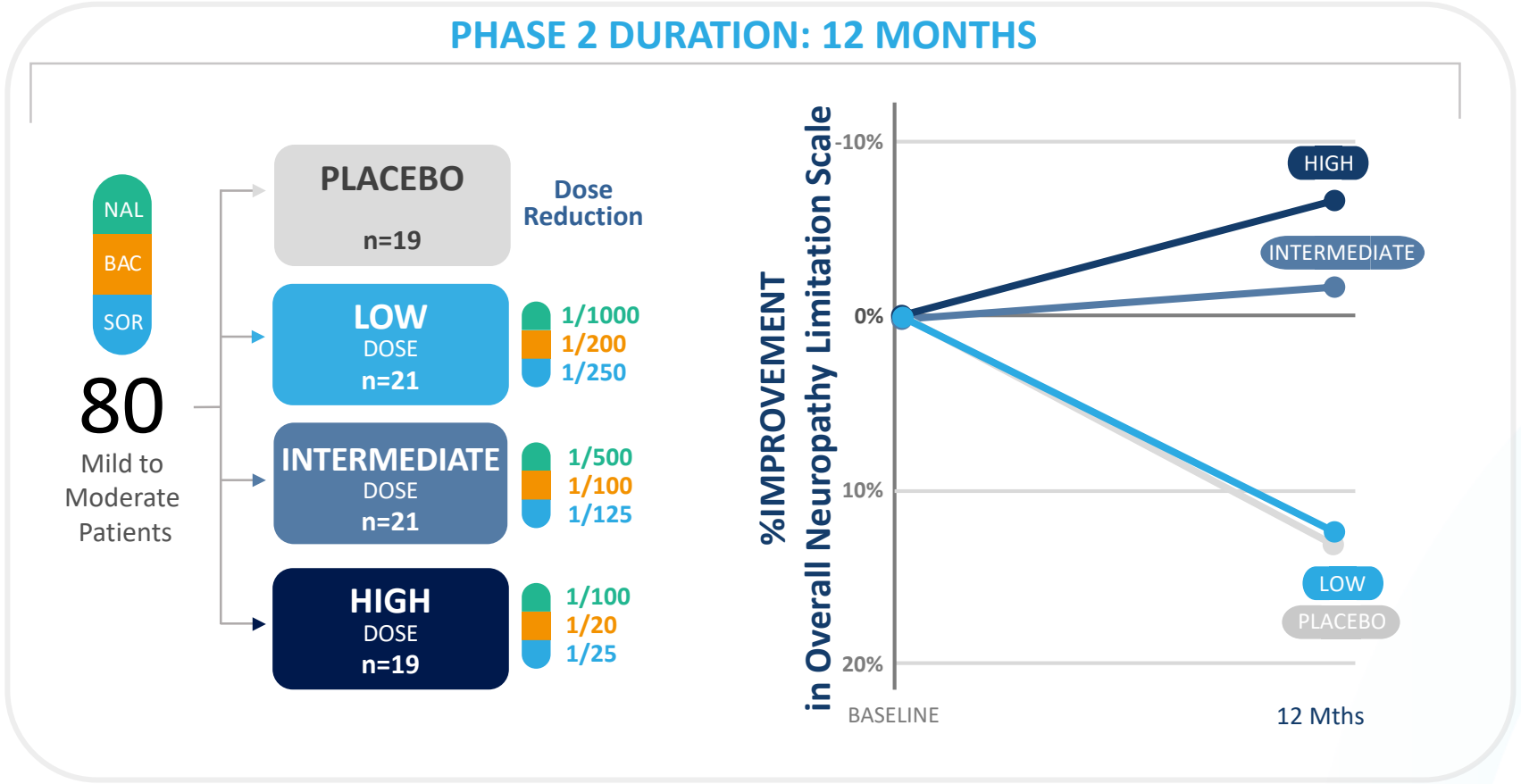


Design of PXT3003

Current Indication		Dose Reduction	
Opioid/ Alcohol dependence	NALTREXONE	Opioid receptor	50mg → 1.4mg
Spasticity	BACLOFEN	GABA receptor	120mg → 12mg
Constipation	SORBITOL	Muscarinic receptor	15g → 420mg

Robust Exploratory Phase 2 Results for PXT3003 in CMT1A

Efficacy and dose-effect demonstrated with Overall Neuropathy Limitation Scale (ONLS) primary endpoint in a multi-center, randomized, double-blind, placebo-controlled Phase 2 study



- All doses safe and well tolerated
- Effect achieved at 12 months with the high dose, which was used to design the Ph3 study

**Attarian et al, Orphanet Journal of Rare Diseases (2014), 9:199*

Abbreviations: ONLS, Overall Neuropathy Limitations Scale.

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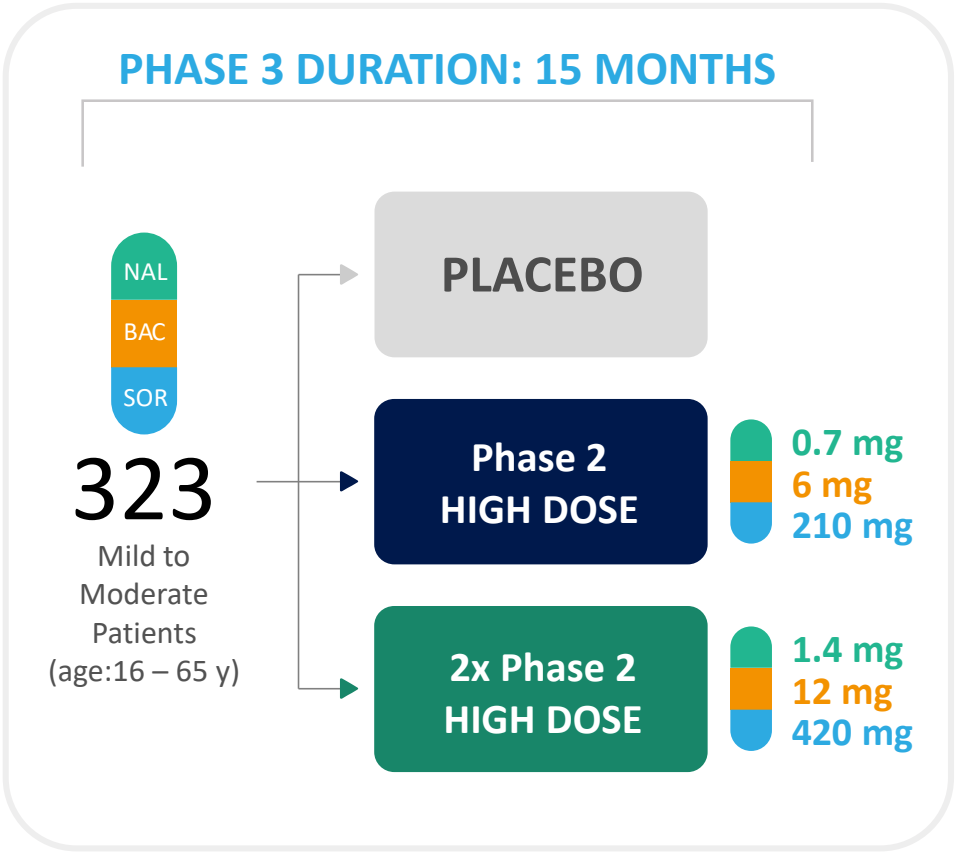
PLEO-CMT: Pivotal Phase 3 Study Design and Endpoints



International, multi-center, randomized, double-blind, placebo-controlled pivotal Phase 3 study

Primary endpoint: Disability measured by the change in ONLS scale in CMT1A patients treated for 12 to 15 months

- ONLS is a 12-point scale evaluating disability
- 90% of the patients in the study had a score between 2-4
- A 0.3 point difference vs. placebo in ONLS was determined to be meaningful*
- FDA and EMA agreed to use ONLS as the primary endpoint for this study

Secondary endpoints analysis include:

- 10 meter walk test (10MWT)
- Nine-hole peg test performed by non-dominant hand
- CMT impairment Score (Clinical and Electrophysiological= CMTNSv2)
  - sensory items**
  - purely clinical items (CMTES)***

* *Cohen J. 1988*
** *Sensory subset of CMTNSv2, items 1,4 and 5*
*** *CMTES is derived from CMTNSv2, items 1 to 7 excluding nerve conductions*

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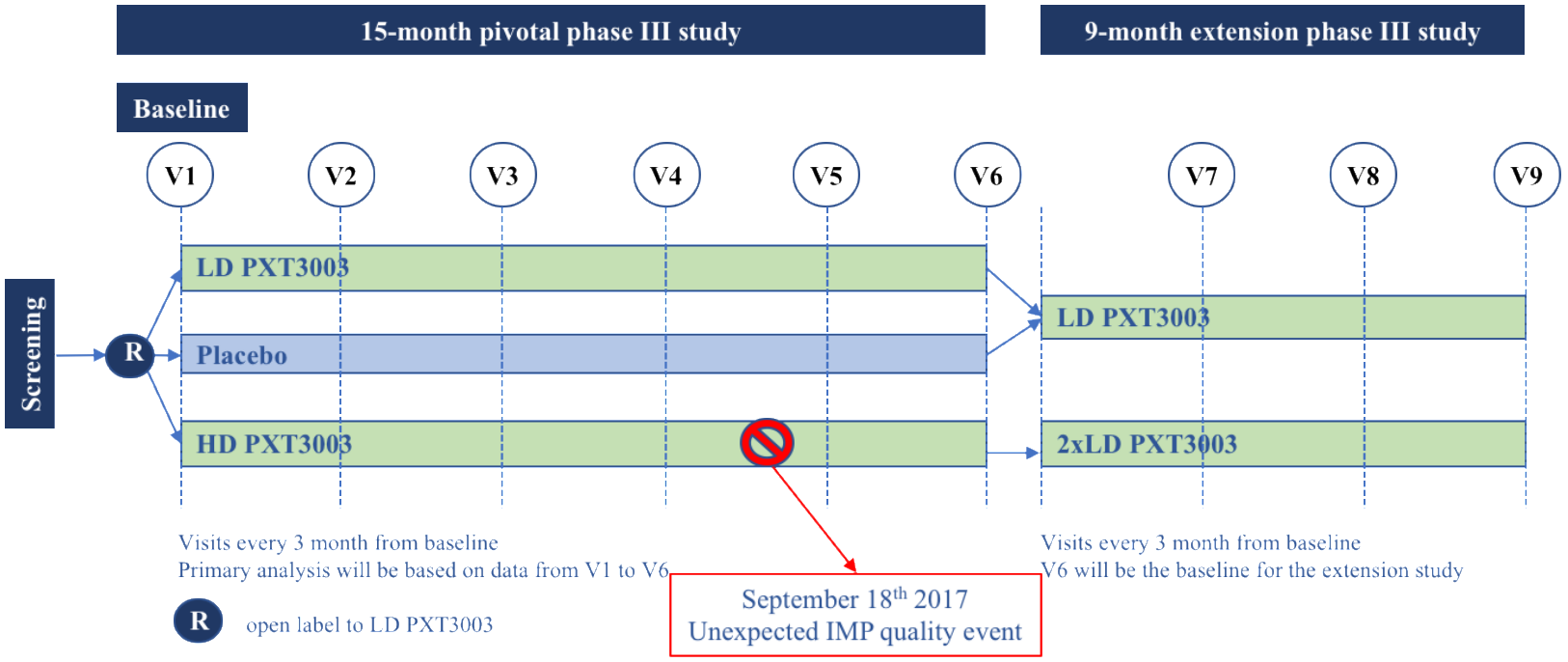
PLEO-CMT: Course of the Study



- Higher dose discontinuation for unexpected formulation / stability issues without safety concern.
- Higher dose patients continued to open label extension study with **2x volume of lower dose.**
- Only blind data were analyzed.

LD: Lower Dose
HD: Higher Dose

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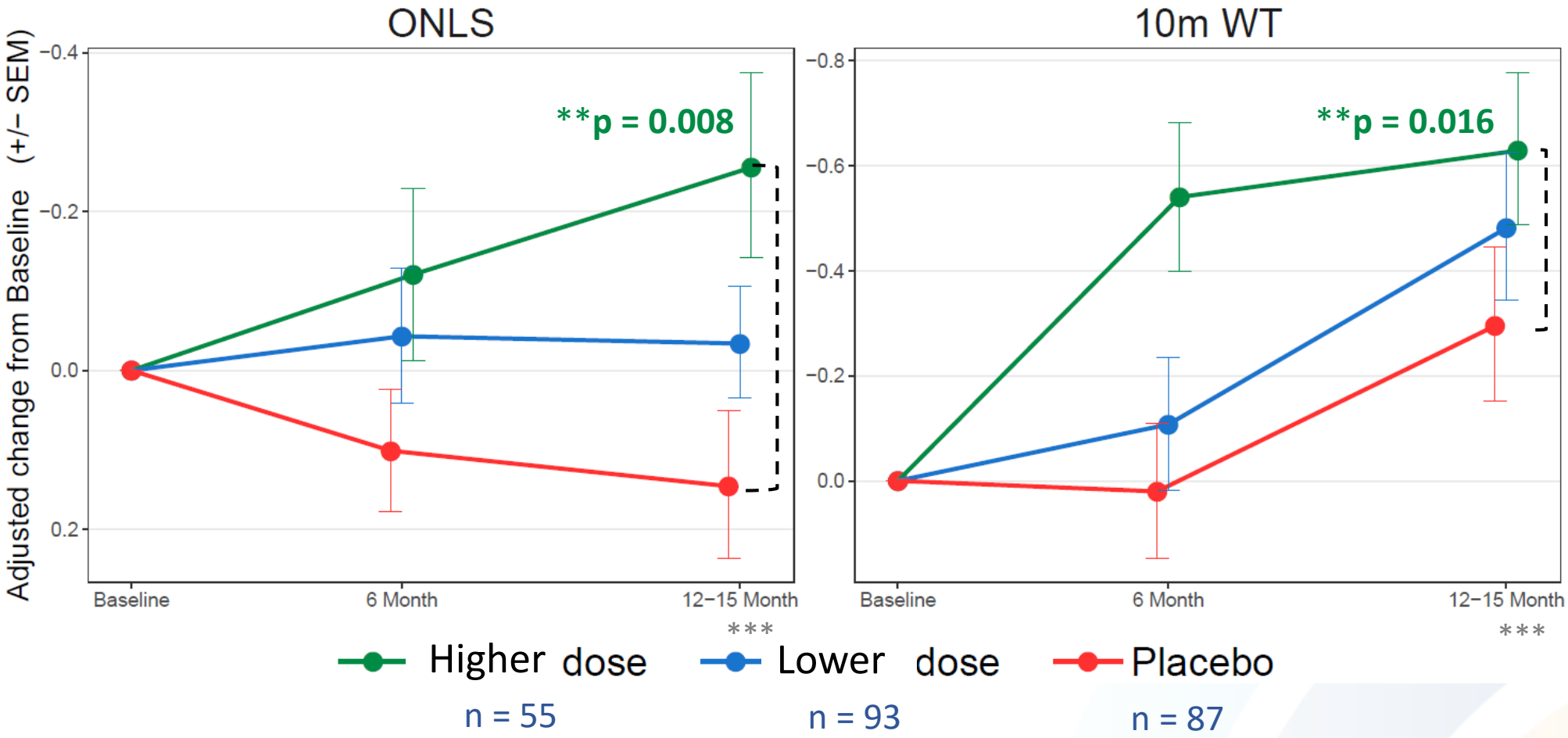
PLEO-CMT: ONLS and 10MWT in the SAP* Primary Population



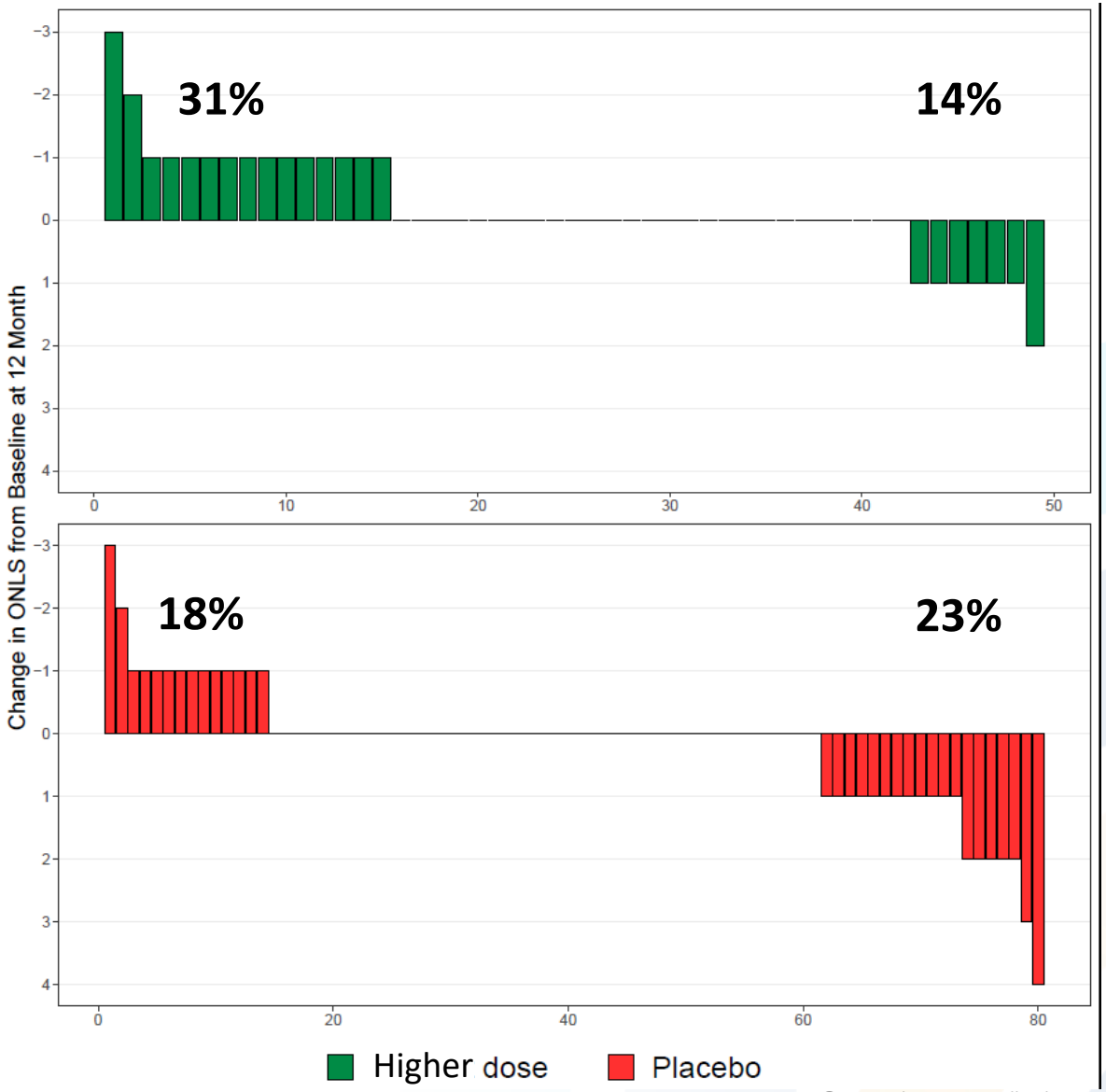
Multiple statistical methodologies supported these compelling positive results

* Statistical Analysis Plan frozen and sent to USFDA before unblinding the data
** Higher dose vs Placebo, ANCOVA with multiple imputation
*** Average of 12 and 15 Month, or 12 Month if 15 Month is missing

PLEO-CMT: Preliminary Individual ONLS Response Analysis*

- Ranked individual ONLS change from baseline for **higher dose** and **placebo**
- **31% higher dose** improved compared to only **18% placebo**
- Only **14% higher dose** declined compared to **23% placebo**

- **PXT3003 higher dose generates BOTH**
  - **Less decline**
  - **Improvement**



** Change from Baseline by Patient Group for 12 Month Completers*

PLEO-CMT: Safety and Tolerability

- Treatment emergent adverse events (TEAE) were similar amongst the three groups and the majority were mild
- TEAEs leading to treatment withdrawal were similar in all three groups
- A single treatment-related serious TEAE occurred in the lower dose group (gastro-enteritis)
- PXT3003 was safe and well tolerated and showed a similar safety profile as seen in Phase 2

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Path Forward

Q4 2018: Clinical Study Report of the pivotal Phase 3

Q1 2019: Type B meeting with FDA

1H 2019: Initiation of Phase 3 pediatric study of PXT3003 in CMT1A

2H 2019: Results of extension study (PLEO-CMT-FU) of the long-term safety and tolerability of PXT3003

2H 2019: Submission of New Drug Application (NDA) to FDA and Marketing Authorization Application (MAA) to EMA

2020: Commercial launch

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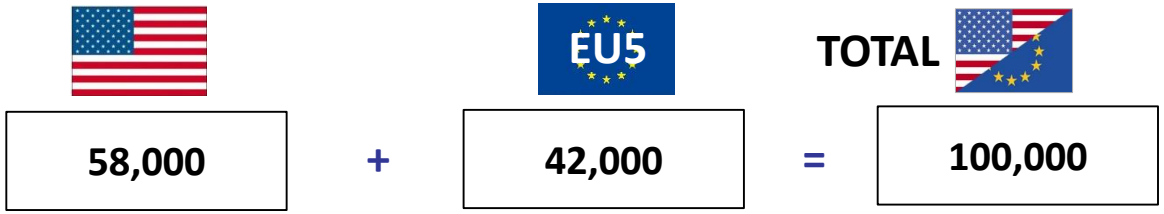
Large Market Opportunity

Significant unmet need – no other treatments currently available for CMT1A

~100,000 mild to moderate CMT1A patients in US and Europe



Initial independent pricing studies show blockbuster potential

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Launch Plan

Commercial Rights

- US, EU, Japan and ROW commercial rights retained by Pharnext
- China rights licensed to Pharnext & Tasly's joint venture

Commercial Strategy

- With these Phase 3 results, Pharnext is strongly committed to prepare filing in Europe and the US
- Pharnext will assess the most adequate commercialization options for various geographical areas

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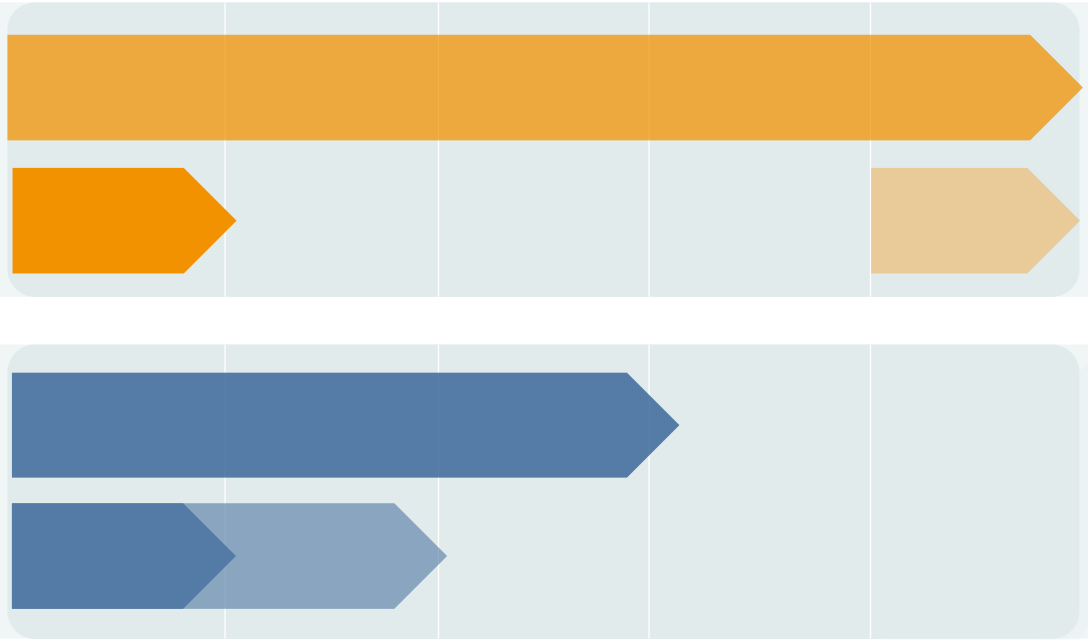
Pipeline and Expected Milestones

Product	Indication	Preclinical	Phase 1	Phase 2a	Phase 2b	Phase 3	Expected Milestone
PXT3003	CMT1A Adults						NDA & MAA submission in H2 2019
	CMT1A Pediatrics						Launch of Phase 3 in H1 2019
PXT864	Alzheimer's disease						Phase 2b initiation expected in 2019
	Amyotrophic lateral sclerosis						POC & Phase 2a initiation expected in 2019



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Summary

- Pharnext, based on the positive pivotal Phase 3 results reported today, intends to file its NDA for marketing approval in 2019 in the US and Europe for a potential launch in 2020
- CMT1A patients have no drug to treat their chronic neuropathy and Pharnext PXT3003 could be the first therapy aimed at treating this severe debilitating disease
- PXT3003 has blockbuster potential
- Having succeeded Phase 3 with its first drug candidate coming from its powerful Pleotherapy platform, Pharnext is committed to developing additional product candidates for other major unmet medical needs

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