Pharming Group N.V.

Proposed Acquisition of Abliva AB

December 16, 2024

NASDAQ: **PHAR** | EURONEXT Amsterdam: **PHARM**

Pharming to Acquire Abliva AB

Strategic Rationale and Offer	Sijmen de Vries, Chief Executive Officer	
KL1333 for mtDNA Mitochondrial Disease	Anurag Relan, Chief Medical Officer	
Commercial Overview	Stephen Toor, Chief Commercial Officer	
Conclusion	Sijmen de Vries, Chief Executive Officer	
Q&A		

Forward-looking statements

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CEO

Sijmen de Vries, MD
Chief Executive Officer

Strategic Rationale and Offer

Building a Leading Global Rare Disease Biopharma Company

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Market RUCONEST® for acute HAE attacks in key markets – U.S. focus

Positive cash flow from RUCONEST® revenue funds Joenja® (leniolisib) launches & pipeline development

- Revenue FY23 US$227.1M
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Global approvals and commercialization of Joenja® (leniolisib) for ADPS

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Ongoing pipeline development and management of rare disease assets

Advance internal projects and rare disease in-licensing and acquisition strategy

- KL1333 for mtDNA mitochondrial disease – pivotal study, significant commercial opportunity, potential first to market end 2028
- Leniolisib for PIDs with immune dysregulation linked to PI3Kδ signalling (Phase II) ~5x APDS pop.
- Undisclosed 3rd PID indication (Phase II planned)

Pending deal close

2024 Total Revenue Guidance - $280 – $295M (14 – 20% growth)

Acquisition Terms and Financial Details

Acquisition Terms

- Acquisition through a public tender offer under Swedish Takeover Act and Nasdaq Stockholm Takeover Rules
- Offer price of SEK 0.45 in cash for each share in Abliva AB, totalling approximately $66M USD *
- Minimum share acquisition target of 90% + 1 share
- Subject to customary regulatory approval

Financial Details

- Acquisition of shares with available cash
- Available cash and future cash flows expected to cover KL1333 development and pre-launch costs and current pipeline investments
- KL1333 in-licensed by Abliva from Yungjin Pharm, which is entitled to milestone and royalty payments **

Timing

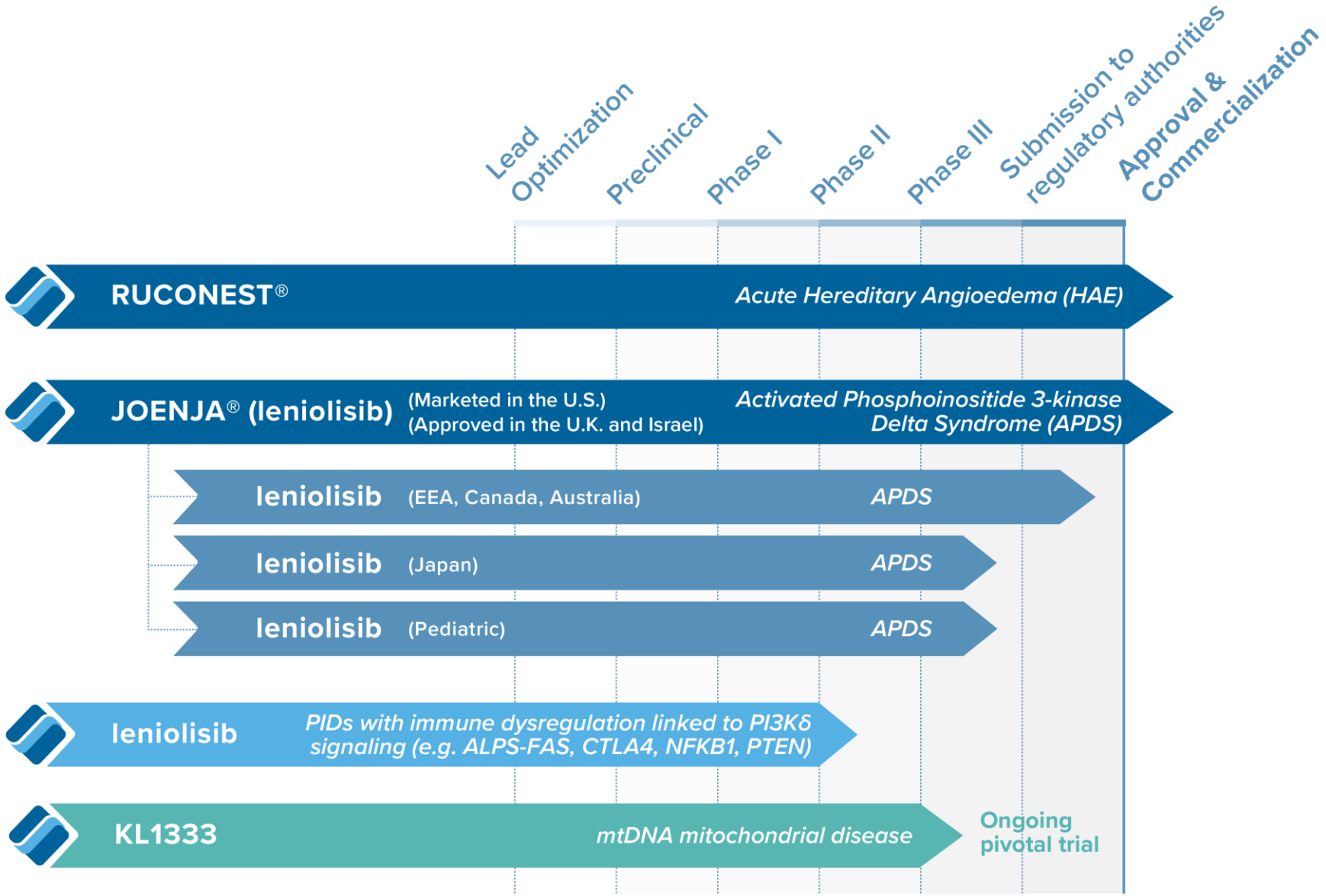
- The acceptance period is expected to commence on or around January 16, 2025, and to expire on or around February 7, 2025

Transaction confirms our strategy of developing a high-value pipeline

*Based on an exchange rate of 0.0911 SEK / USD from 13 December 2024

**Worldwide rights, excl. Japan and South Korea primarily for the treatment of genetic mitochondrial disease; single-digit to low double-digit royalties on net sales, plus development and commercial milestone payments

KL1333 Adds Value to Pharming's Pipeline



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Anurag Relan, MD

Chief Medical Officer

KL1333 for mtDNA mitochondrial disease

Attractive Rare Disease Opportunity Fits Pharming's Business Model

Mitochondria are the "powerhouses" of cells

- Producing chemical energy to power the human body, in particular muscle cells

Primary mitochondrial diseases are rare disorders affecting the mitochondria's ability to generate energy

- Patients suffer from severe fatigue, myopathy (muscle weakness) and reduced life expectancy
- Debilitating symptoms where patients cannot lead normal lives (e.g., loss of job, social isolation, depression)

KL1333 is a novel, first-in-disease therapy using a mechanism of action that addresses the underlying disorder

- Targets mitochondrial DNA (mtDNA) driven primary mitochondrial diseases with >30,000 diagnosed patients in US, EU4 & UK

Pivotal study is ongoing with positive interim analysis confirming the FDA-agreed primary endpoints

- Read-out anticipated in 2027 with potential FDA approval by end of 2028

Attractive commercial opportunity with significant unmet medical need and no approved therapies

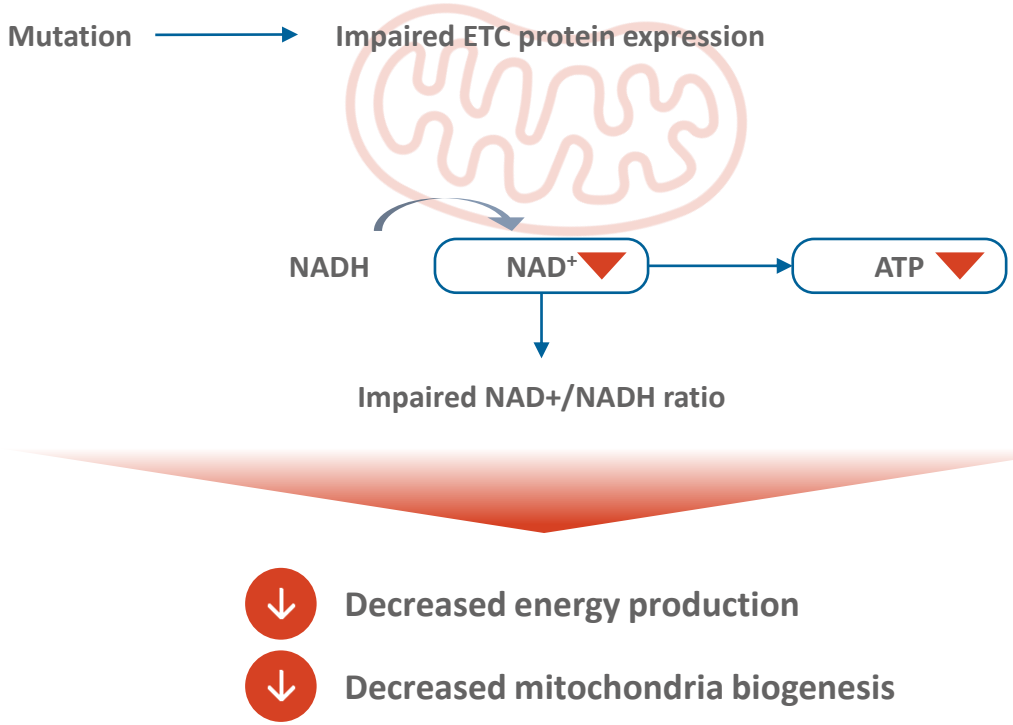
- Builds on Pharming's expertise to bring novel, life-changing rare disease therapies to patients
- Enables the ability to leverage Pharming's existing rare disease commercial infrastructure

Dysfunctional Mitochondria Produce Less ATP

Primary Mitochondrial Disease (PMD)

- Mitochondria, often described as the “powerhouses” of cells, are crucial for energy production
- Mitochondrial diseases are a group of genetic disorders characterized by dysfunctional mitochondria due to mutations in mitochondrial (mtDNA) or nuclear DNA
- The abnormal NAD^+/NADH ratio results in decreased ATP production, contributing to organ dysfunction and disease deterioration
- For patients this means symptoms of severe fatigue and muscle weakness – symptoms which patients report as the most troublesome*

Dysfunctional Mitochondria



*Voice of the Patient Report, United Mitochondrial Disease Foundation, 2019.

NAD: Nicotinamide adenine dinucleotide; NADH: Nicotinamide adenine dinucleotide + hydrogen; ETC: Electron transport chain.

Heavy Patient Burden with No Approved Therapies

Presentation and Diagnosis

- Patients present to their primary care doctor and then often get referred to a neurologist for musculoskeletal issues
- Either the neurologist or a referral to a metabolic geneticist will result in a diagnosis
- Many patients are diagnosed at academic centers specializing in mitochondrial disease
- A combination of routine lab tests and genetic testing available from major testing labs help to diagnose patients

Impact

- Patients heavily burdened in their daily lives including symptoms like severe fatigue, myopathy, and metabolic dysfunction
- Impact on QoL including loss of job, loss of independence, depression/anxiety
- Primary mitochondrial diseases lead to a three-to-four-decade reduction in life-expectancy

Treatment

- No approved treatment options
- Patients are limited to using vitamins, supplements, and physical therapy

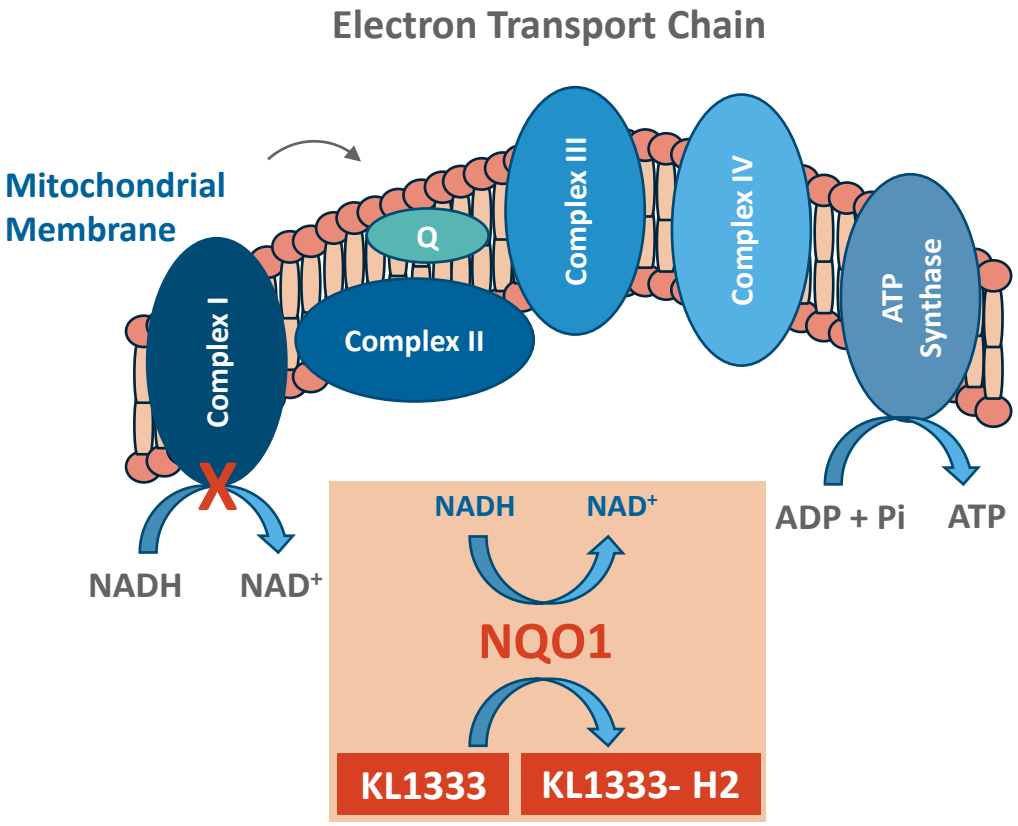
" On the worst days I will be crying in frustration because going to the kitchen seems equivalent to climbing a mountain and just trying to process what others are saying to me involves all the energy and concentration that I have."

United Mitochondrial Disease Foundation, Voice of the Patient Conference, 2019

KL1333 Corrects the Underlying Pathophysiology

KL1333 normalizes conversation of NADH to NAD+ via NQO1

Normalizes the NAD⁺/NADH Ratio



Restored Energy Metabolism

- Restored energy regulation and improved ETC function
- Stimulation of mitochondria biogenesis
- Overall resulting in symptom reduction and expected disease modification

KL1333: First-in-Disease Small Molecule with Unique MOA

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Attributes

- Directly increases the NAD+/NADH ratio via NQO1
- Unique MoA works upstream from all competing MoA in PMD
- Oral, small molecule, BID dosing
- Favourable safety profile
- Favourable IP protection
- Orphan Drug Designation in US & EU and FDA Fast Track
- Potential first-in-disease with registrational clinical study

Outcomes

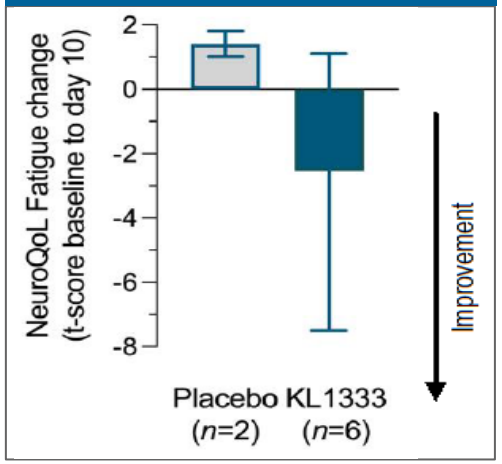
- Improved energy regulation and ETC function
- Stimulation of mitochondria biogenesis
- Fatigue reduction
- Increased exercise capacity

Phase 1b Demonstrated Significant Activity vs. Placebo

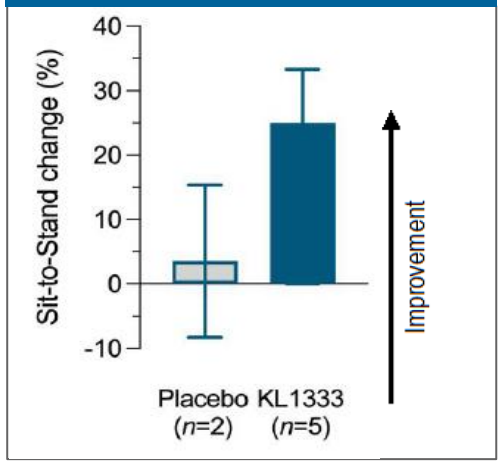
The placebo-controlled Phase 1b study demonstrated that KL1333 reduced patients' fatigue and myopathy after only 10 days, 50 mg/day

- KL1333 demonstrated efficacy in the phase 1b placebo-controlled portion with patients diagnosed with mtDNA mitochondrial disease
  - Fatigue reduction (NeuroQoL fatigue change)
  - Muscle function improvement (30 seconds sit-to-stand)
- KL1333 showed efficacy signals after 10 days using 50 mg/day
- Mitochondrial patients have increased lactate levels and increasing the concentration of KL1333 resulted in an improved lactate/pyruvate ratio, reflecting target engagement
- No serious adverse events reported

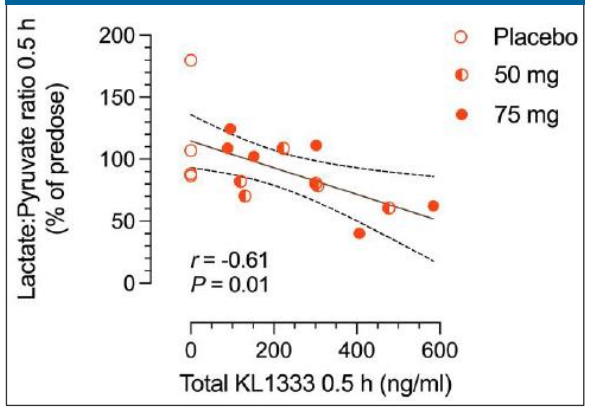
Fatigue Reduction
Changes from baseline to day 10



Muscle Function Improvement
Changes from baseline to day 10



Lactate:Pyruvate Ratio
Changes from baseline to day 10



Source: Pizzamiglio C et al., Optimizing rare disorder trials: a phase 1a/1b randomized study of KL1333 in adults with mitochondrial disease, Brain, 2024.

Pivotal study Design Based on Regulatory and Patient Advocacy Input

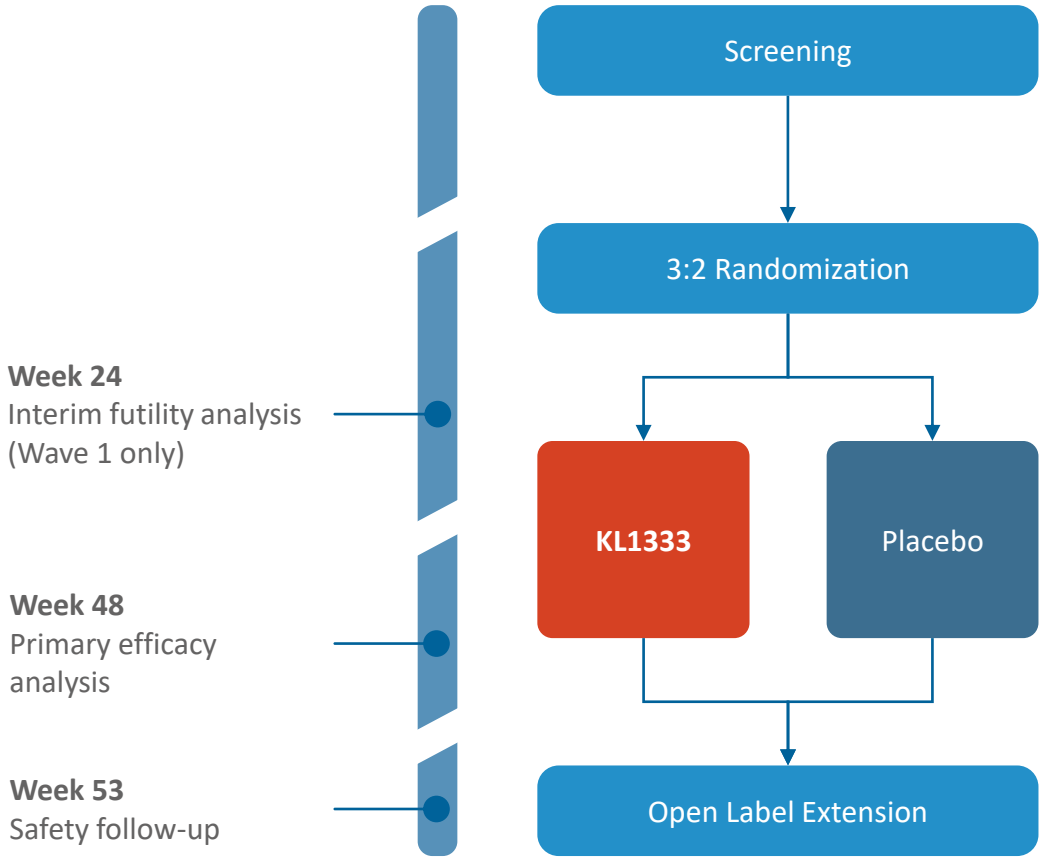
Regulatory Feedback

- Both FDA and EMA accepted study as registrational
- FDA said achieving one of the two endpoints would be sufficient for filing
- Conducted regular and detailed discussions with the FDA to facilitate alignment

Study Design

- **Methodology**
  - Randomized, double-blind, parallel-group, placebo-controlled pivotal study
- **Patients Included**
  - Adult PMD patients with mtDNA mutations* with fatigue and myopathy
- **Primary Endpoints**
  - Fatigue using the PROMIS Fatigue Mitochondrial Disease Short Form
  - Muscle weakness using the 30 second Sit-to-Stand test

Study Schematic



*Most prevalent mtDNA disorders include m.3243A>G associated MELAS-MIDD spectrum disorders, single large scale mtDNA deletion associated KSS-CPEO spectrum disorders, other multisystemic mtDNA-related disease (including MERRF)

FALCON Study – Positive Interim Analysis

Pivotal FALCON Study

WAVE 1 – Fully enrolled

- 40 patients recruited across six countries (U.S., UK, France, Spain, Belgium, Denmark)
- 18 sites activated
- Interim analysis at 24 weeks conducted in Q3 2024

WAVE 2 – Expansion

- 180 total patients treated for 48 weeks
  - Wave 1 sites ready to start enrolling
  - Wave 2 sites undergoing activation
- Readout anticipated 2027

Interim Futility Analysis:

Positive outcome achieved, with both primary endpoints having passed futility

- Promising differences favoring the active arm vs. placebo for both primary efficacy endpoints; if trends continue consistently, we expect a successful result at the completion of this trial
- Data monitoring committee (DMC) recommended continuing with Wave 2:
  - Safety and tolerability profile acceptable
  - No changes to study design
  - 180 total patients confirmed in the study

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CCO

Stephen Toor

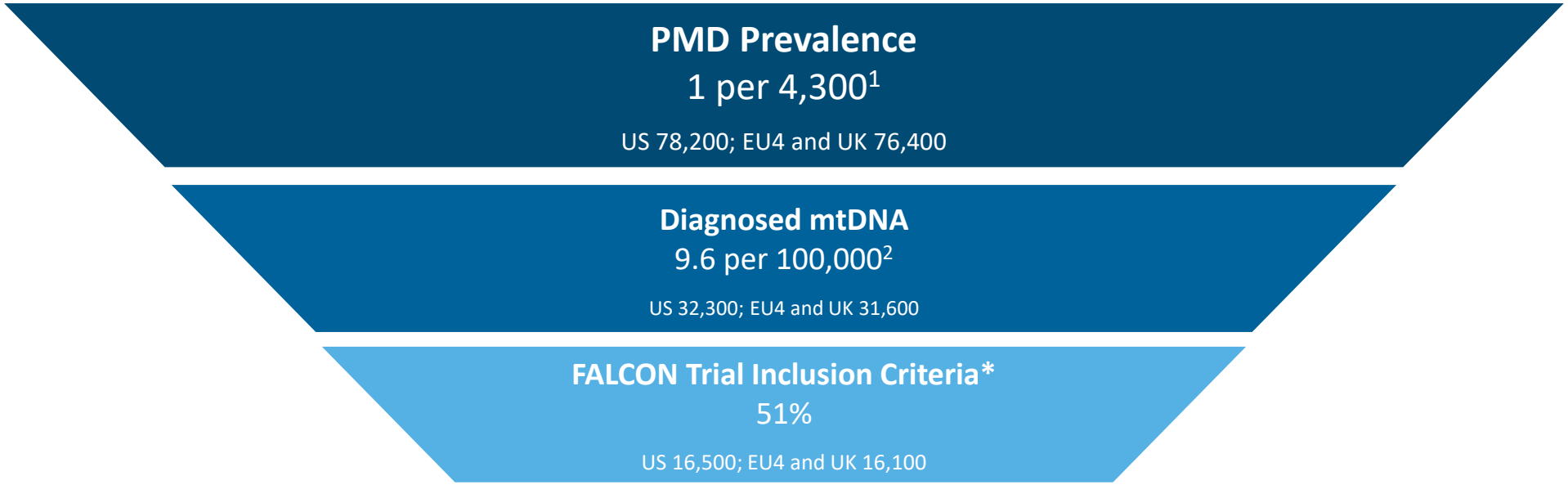
Chief Commercial Officer

Commercial overview

First-In-Disease Rare Disease Therapy with Blockbuster Potential

- Builds on Pharming's existing rare disease expertise and infrastructure
- **KL1333 positioned to become first standard of care in mtDNA mitochondrial disease**
- **Large commercial opportunity with >30,000 mtDNA diagnosed patients in US, EU4 and UK**
- Concentrated centers of excellence and strong advocacy groups
- Small molecule tablet has low COGS and easy route of administration

Significant Revenue Opportunity for KL1333

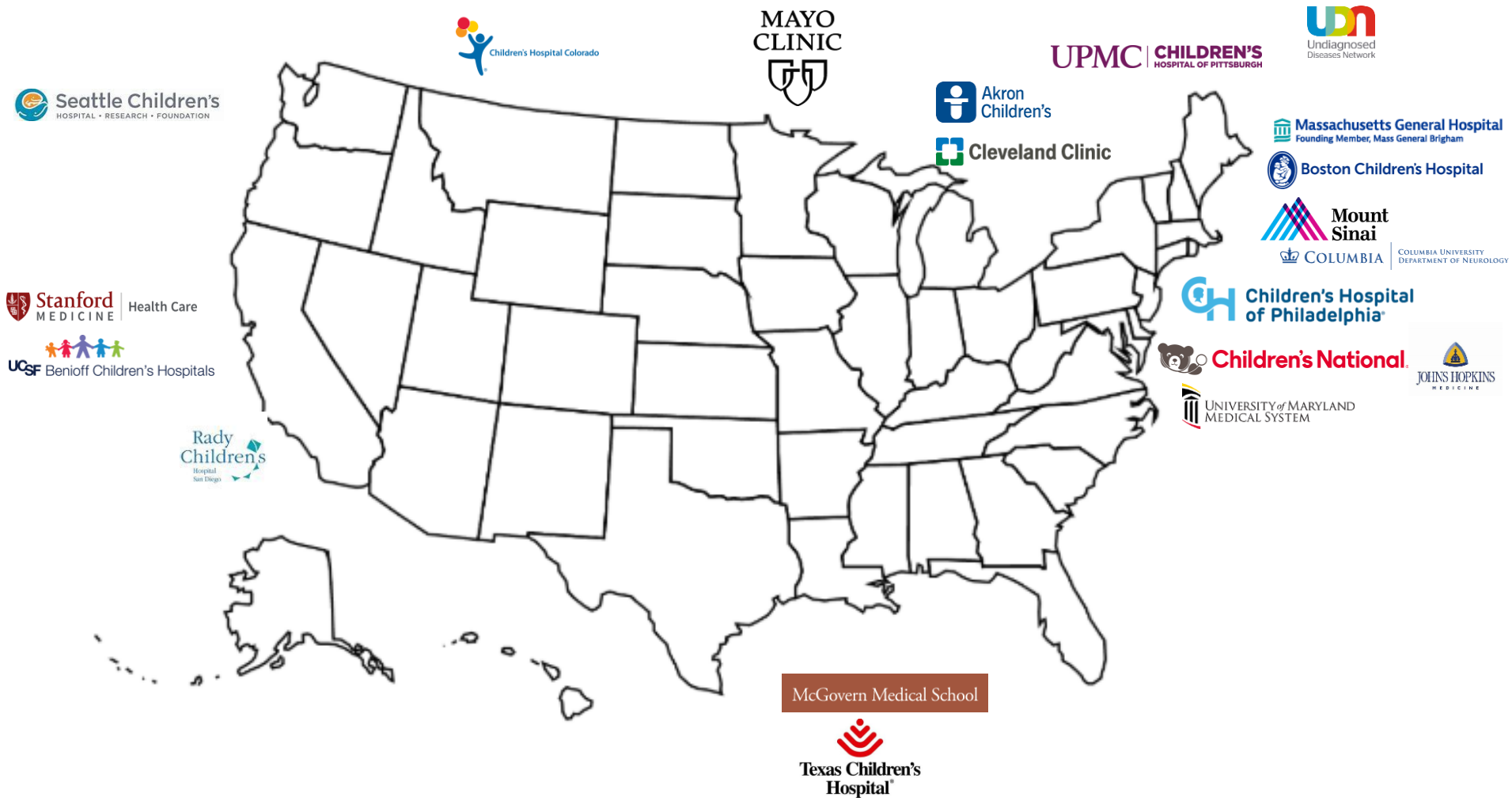


>30,000 diagnosed mtDNA mitochondrial disease patients addressable in the US, EU4 and UK

*mtDNA mutations including m.8344A>G MELAS-MIDD, MERRF, KSS-CEPO, large scale mtDNA deletions

[1] Gorman, G.S. et al. Prevalence of nuclear and mitochondrial DNA mutations related to adult mitochondrial disease. Ann Neurol 2015 May;77(5):753-9.
[2] Gorman, G.S. et al. Mitochondrial Diseases. Nat. Rev. Vol 2, 1-22 (2016).

Majority of Patients Diagnosed and Treated in US Centers of Excellence or Academic Institutions



Mitochondrial Care Network – Centers: www.mitonetwork.org/centers

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Conclusion

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