Targeting the powerhouse of cells to improve the lives of primary mitochondrial disease patients

RedEye Investor Seminar

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ABLIVA

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We are….

… a **team of experienced drug developers** located in Lund, Sweden and Boston, MA, USA

… a biotech company with **full R&D capabilities** and a plan to commercial our drugs ourselves

… proud of our portfolio of first-in-class assets to treat **Primary Mitochondrial Diseases (PMD)**

- Rare disease indications with regulatory opportunities (ODD designation) and blockbuster sales potential

… publicly traded on NASDAQ Sweden (**ABLI**, small cap, ~270M SEK market cap)

…Creating the foundation to become the global leader in mitochondrial medicine

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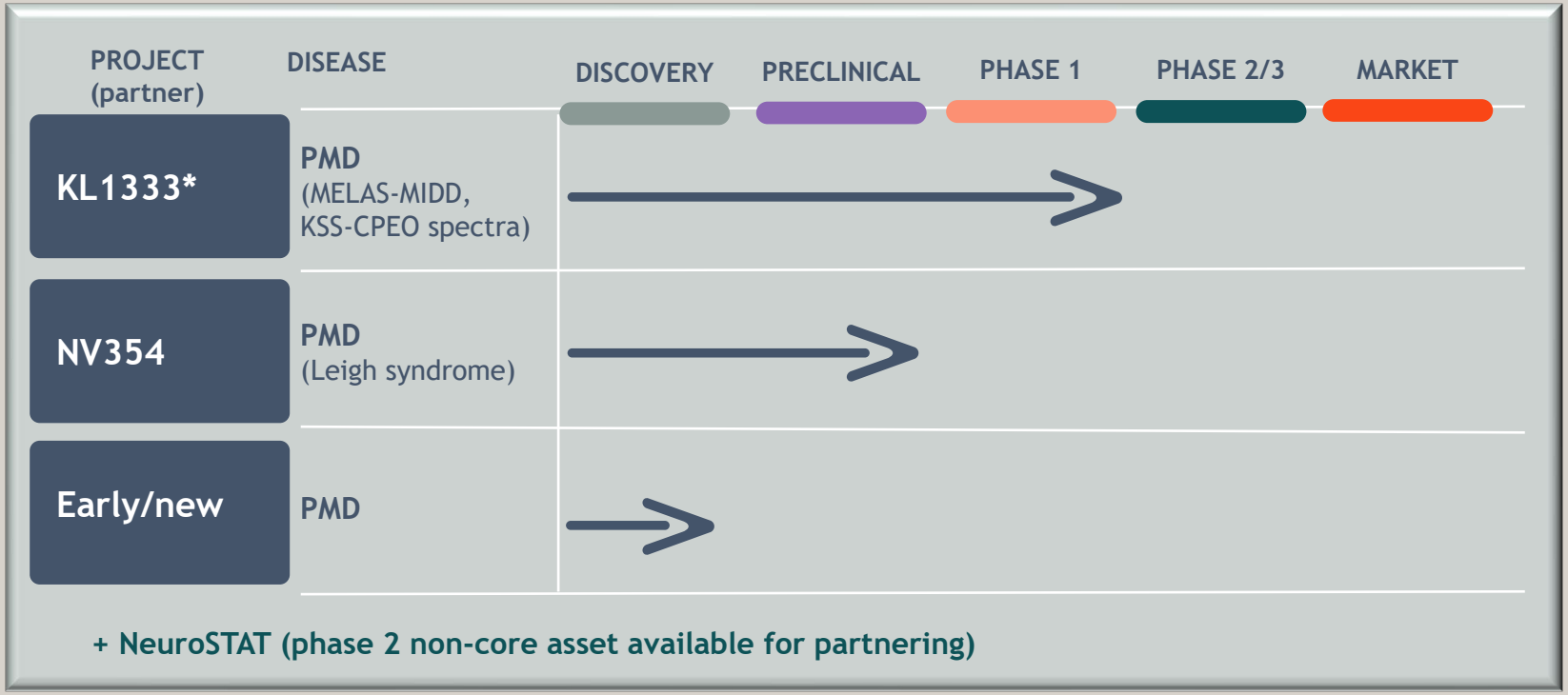
A portfolio of first-in-class therapies targeting underlying pathology in Primary Mitochondrial Diseases (PMD)



*Orphan drug designation in the US and Europe

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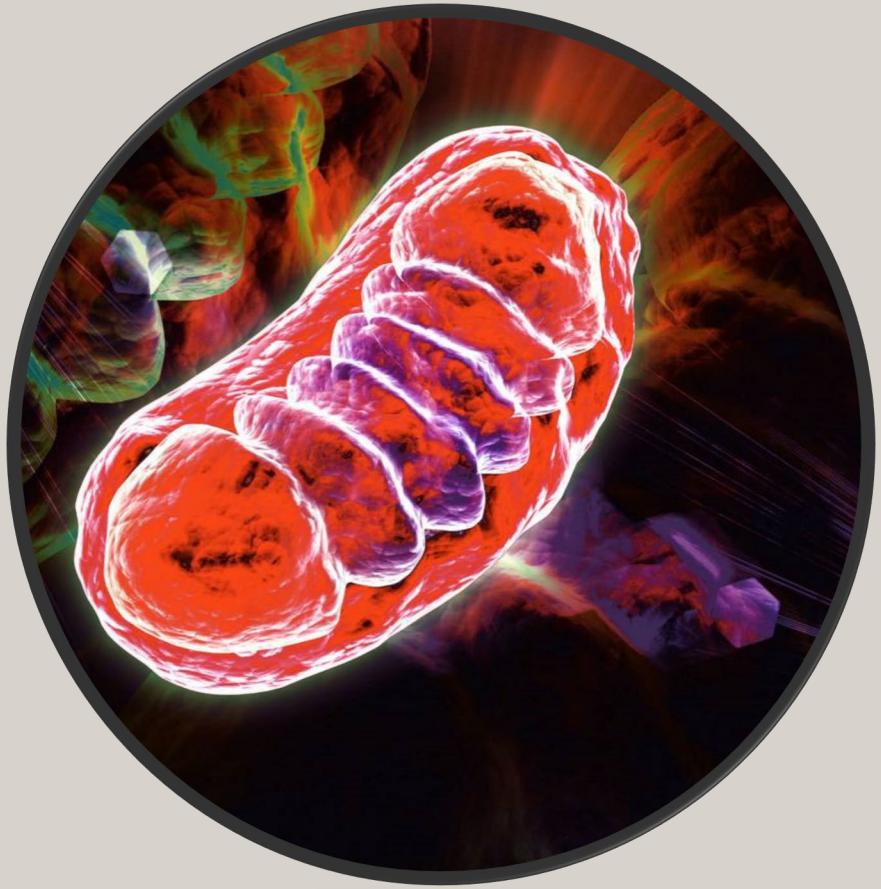
What are primary mitochondrial diseases (PMD)?

- Devastating, rare diseases with severe symptoms and continuous deterioration
  - Affects 1 in 5,000 individuals
- Patients suffer from fatigue, myopathy and multi-systemic complications
- No approved medicines for systemic PMD
- DNA defects cause abnormal oxidative phosphorylation, defective cellular energy production
  - Disruption in NAD+/NADH ratio



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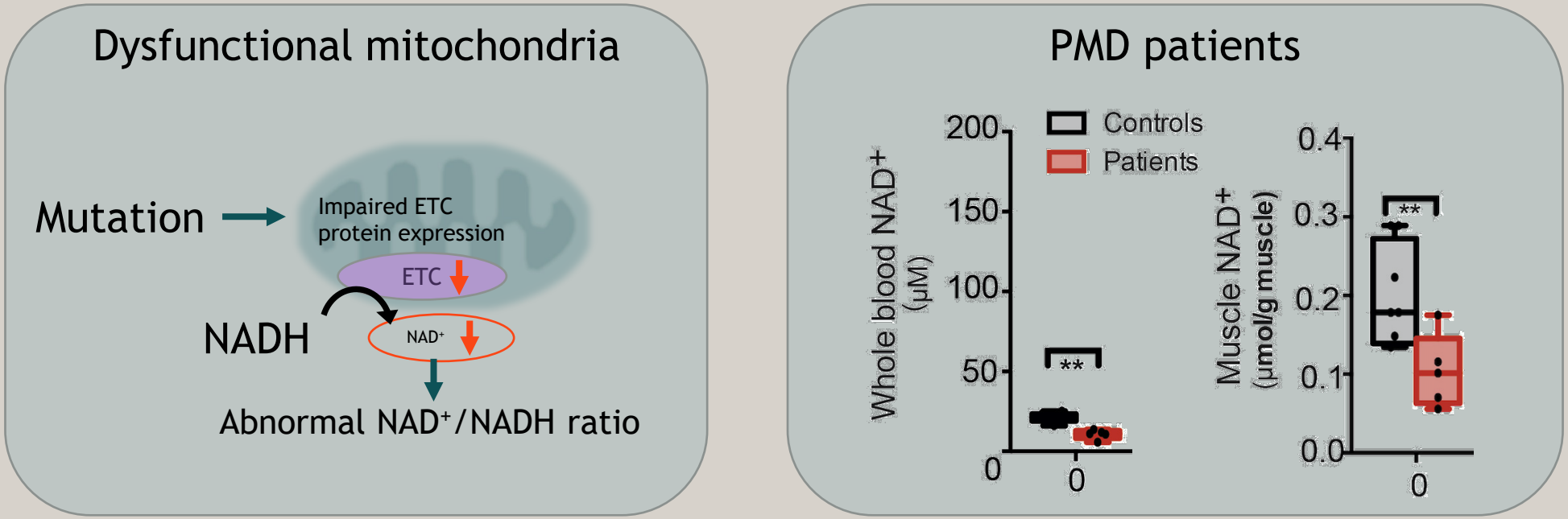
Dysfunctional mitochondria and PMD patients have disrupted generation of NAD+



Result? Lack of energy followed by organ dysfunction and disease deterioration

Pirinen et al. Cell Metab. 2020;31(6):1078-1090

NAD= Nicotinamide adenine dinucleotide, NQO1= NAD(P)H quinone dehydrogenase oxidoreductases 1, ETC=Electron Transport Chain

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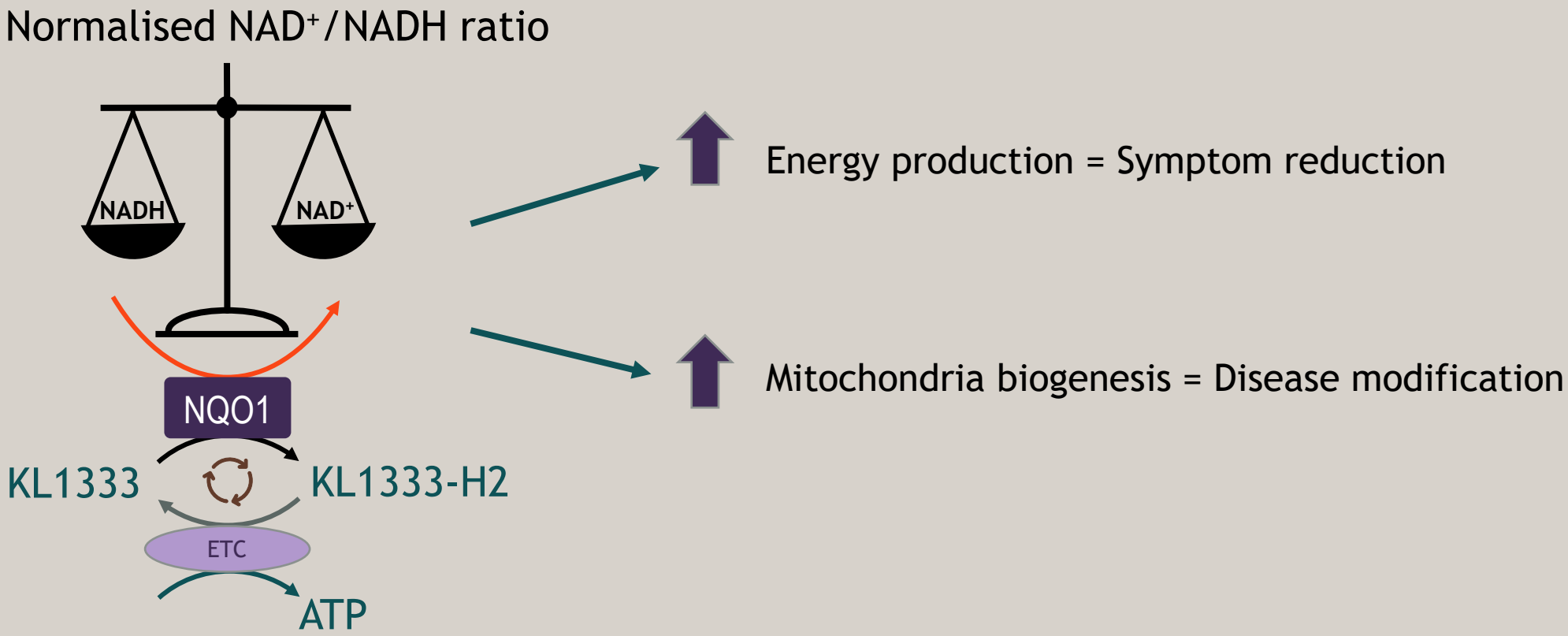
KL1333 corrects underlying pathophysiology of mito disease



NAD= **Nicotinamide adenine dinucleotide,** NQO1= NAD(P)H quinone dehydrogenase oxidoreductases 1, ETC=Electron Transport Chain

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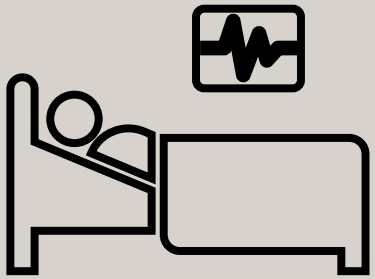
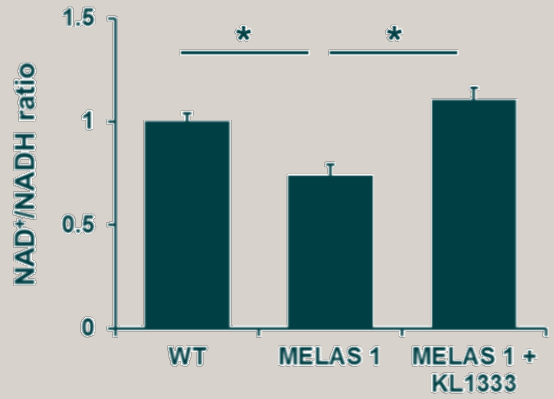
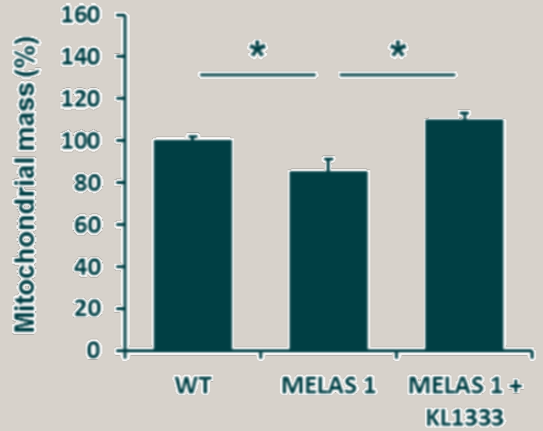
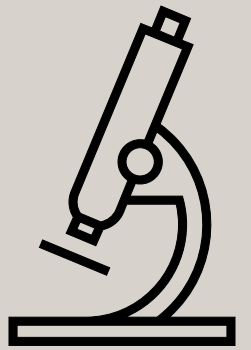
KL1333 Mechanism of action confirmed

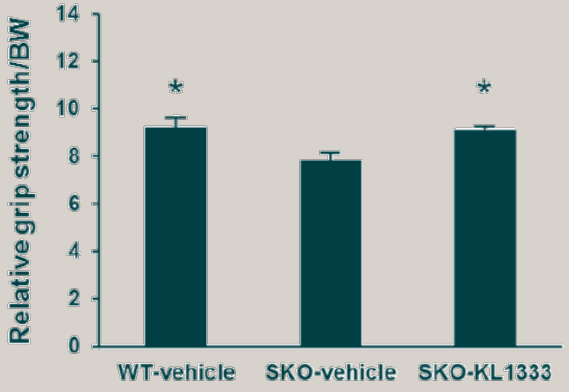


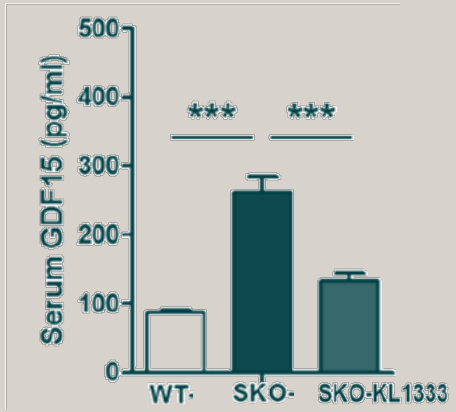




Improves NAD+/NADH ratio and mitochondrial mass in patient fibroblasts







Demonstrates functional and biomarker improvements in vivo

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Phase 1a/1b study design

What? A Randomised, Double-blind, Parallel-group, Placebo-controlled, Phase Ia/Ib, Multiple-site Study

Why? Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of KL1333 after a

How? Single Oral Dose and Multiple Ascending Oral Doses

Who? Healthy Subjects and Patients with Primary Mitochondrial Disease

Study contained 4 parts, with 56 healthy volunteers and 8 PMD patients:

- Part A: SAD with Food effect, 1 cohort 25 mg QD, healthy volunteers
- Part B: MAD, 5 cohorts 25, 50, 75, 150 and 250 mg QD, healthy volunteers
- Part C: 10 days dosing, 50 mg QD, 1 cohort, PMD patients
- Part D: Split dosing (75 mg BID or 50 mg TID), 2 cohorts, healthy volunteers

Healthy volunteer cohorts began in March 2019 and were run at the Covance CRU in Leeds, UK.

Patient cohort was started in October 2020 and was run at University College London (Robert Pitceathly, Chief Investigator) and Newcastle (Grainne Gorman), UK.

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Phase 1a/1b objectives

Primary	▪ **Safety and tolerability of a single oral dose** (with/without food) in healthy volunteers (HVs) ▪ **Safety and tolerability of multiple oral doses** in HVs and PMD patients
Secondary	▪ **Single oral dose plasma pharmacokinetics (PK)** in HVs, including the effect of food intake ▪ **Multiple oral dose plasma PK** in HVs and PMD patients
Exploratory	▪ Multiple-dose PD in HVs and PMD patients using blood **biomarkers** ▪ **Clinician- and patient-rated outcome assessments** following multiple oral doses of KL1333 in PMD patients

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The impact of KL1333 on fatigue was assessed using two independent scales

Scales:

- Quality of Life in Neurological Disorders (Neuro-QoL) Short Form (SF) Fatigue
- Daily Fatigue Impact Scale (D-FIS)

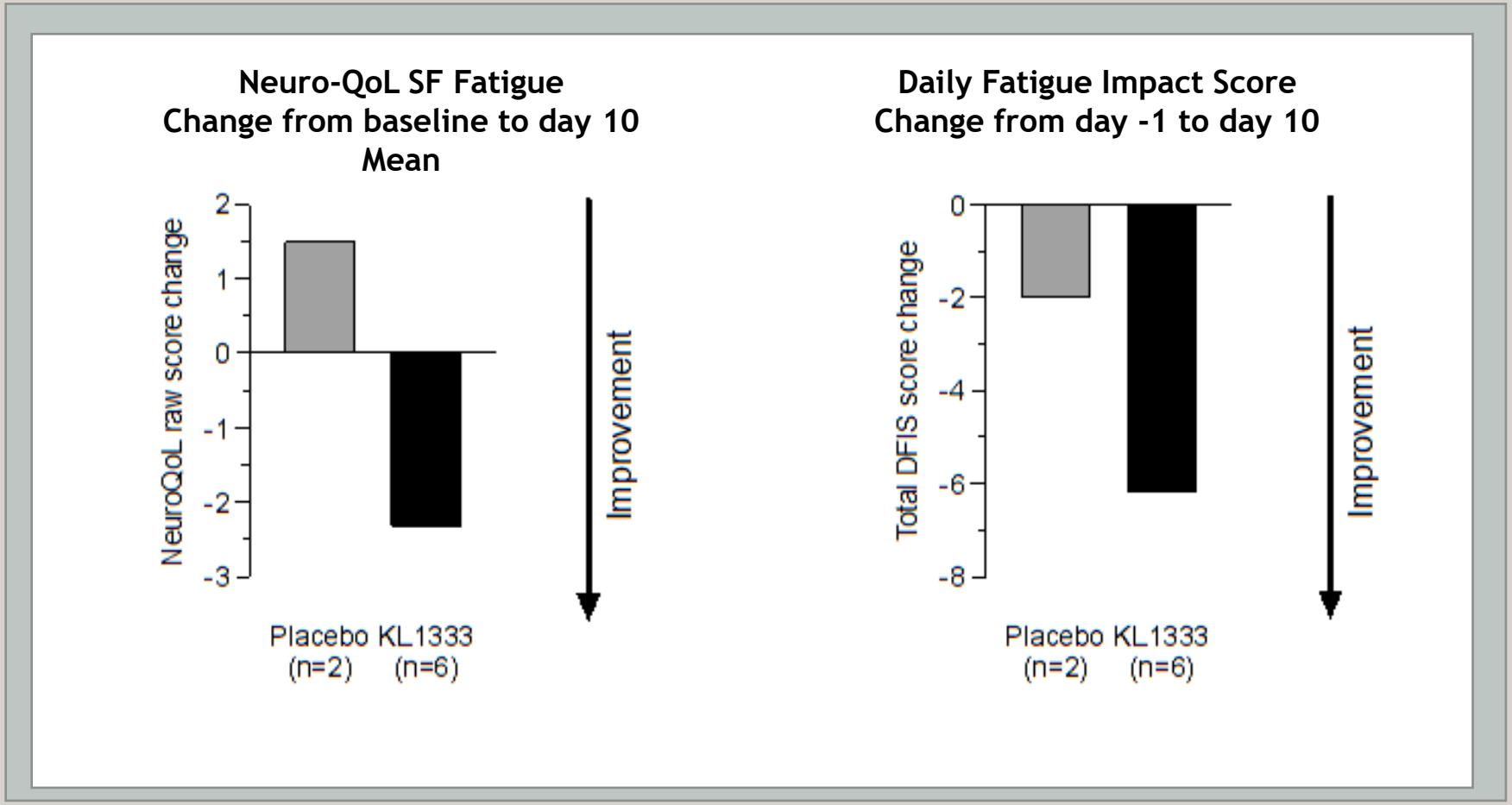
Relevance:

- Fatigue is critically important to patients
- Fatigue endpoint will be primary endpoint in the Phase 2/3 study
  - Supported by the FDA
  - Neuro-QoL fatigue is equivalent to the PMD-specific form now being validated by Abliva

Neuro-QoL Short Form Fatigue

In the past 7 days…	Never	Rarely	Sometimes	Often	Always
I felt exhausted……	☐ 1	☐ 2	☐ 3	☐ 4	☐ 5
I felt that I had no energy……	☐ 1	☐ 2	☐ 3	☐ 4	☐ 5
I felt fatigued……	☐ 1	☐ 2	☐ 3	☐ 4	☐ 5
I was too tired to do my household chores.	☐ 1	☐ 2	☐ 3	☐ 4	☐ 5
I was too tired to leave the house……	☐ 1	☐ 2	☐ 3	☐ 4	☐ 5
I was frustrated by being too tired to do the things I wanted to do……	☐ 1	☐ 2	☐ 3	☐ 4	☐ 5
I felt tired……	☐ 1	☐ 2	☐ 3	☐ 4	☐ 5
I had to limit my social activity because I was tired……	☐ 1	☐ 2	☐ 3	☐ 4	☐ 5

Patients given KL1333 showed a marked improvement in fatigue with only 10 days of dosing.



Minimally important differences for fatigue scales ranges from 2.5-5 in T-score (Yost et al 2011);
DFI-S ranges from 3-4 as extrapolated from Nordin et al. 2016.

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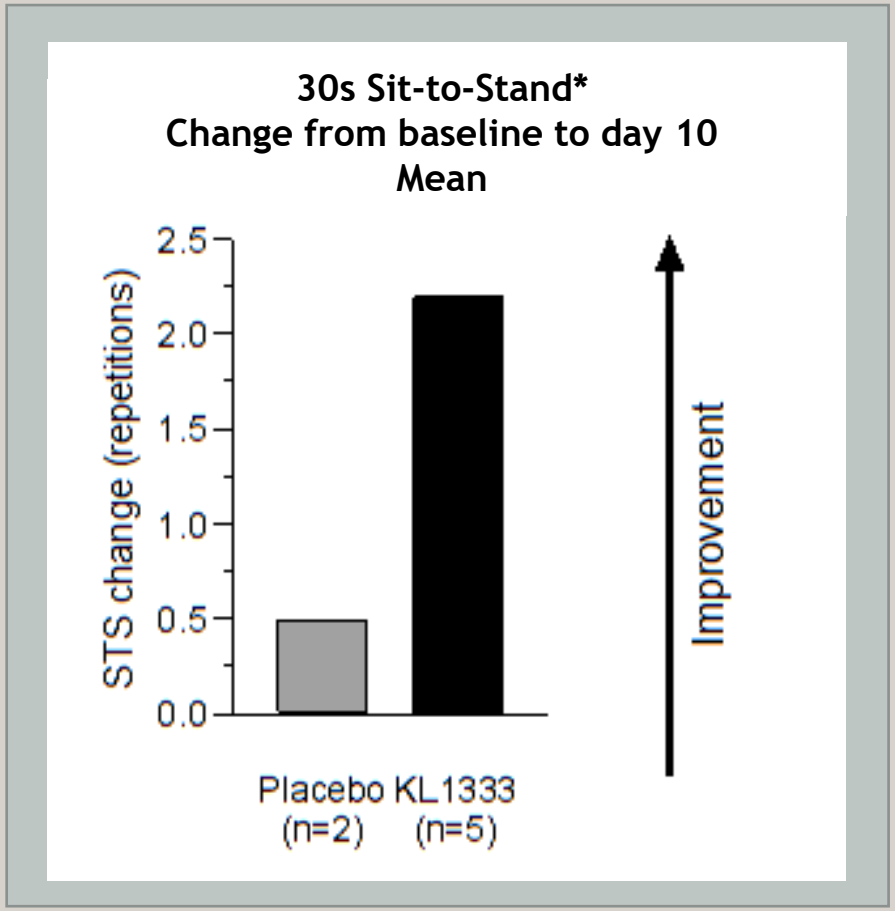
The 30 sec Sit-to-Stand endpoint also showed signs of efficacy in PMD patients.



Notes on Sit-to-Stand:

- Score = number of repetitions (higher score = better muscle strength/endurance)
- ≥2 repetitions has been defined as the minimum clinically important difference in osteoarthritis and COPD**

* One subject in the KL1333 group did not perform test (excluded from analysis)
**Wright et al 2011 (Osteoarthritis), Zanini et al. 2019 (COPD)

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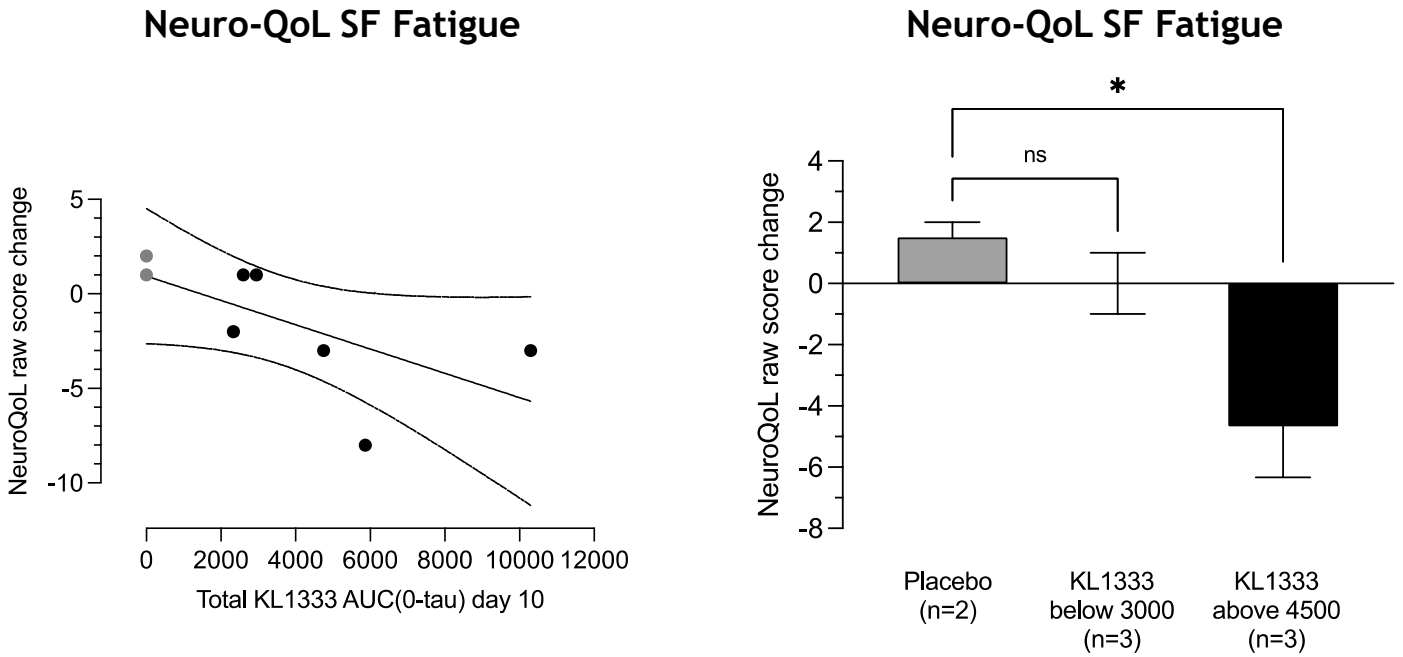
Exposure and effect were correlated across these clinical outcome measures

Reduction of fatigue in relation to exposure of KL1333



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What is the impact of this data on the KL1333 program?

- Provides the **first evidence of efficacy of KL1333** in PMD patients
  - **Clinically-relevant endpoints** that will be used in the upcoming Phase 2/3 study

- **Further strengthens the use of fatigue as an endpoint**
  - Supports the **inclusion of 30 sec Sit to Stand** (or comparable functional endpoint)

- This study, in addition to the drug-drug interaction study that just read out, confirm a **good safety profile** for KL1333 with main dose-limiting tolerability of gastrointestinal side effects

The placebo-controlled nature of the cohort, the strong signal over a small number of patients and the association between exposure and effect give us confidence moving into the Phase 2/3 study.

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The KL1333 program, now stronger, remains on track for the 2021 milestones

KL1333

- ✓ Phase 1a/b study results (H1 2021)
- ✓ Drug-drug interaction study results (H1 2021)
- Fatigue endpoint validation (H2 2021)
- Regulatory submission (H2 2021)
- Regulatory approval (H2 2021)
- Phase 2/3 efficacy study start (H2 2021)

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