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

Abliva Corporate Intro

February 1, 2023

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Abliva is focused on becoming a global leader in mitochondrial medicine

Experienced team with 20+ years in mitochondrial research and drug development

- Offices in Lund (Sweden) and Boston (US)

Full **R&D capabilities** with a plan to **build to commercialize lead asset**

Portfolio of first-in-class clinical assets to treat **Primary Mitochondrial Diseases (PMD)**

- Lead asset, KL1333, is currently in pivotal study
- NV354 is ready for Phase 1

Publicly traded on NASDAQ Sweden (ABLI, small cap)

- 24 months of runway with raise in June 2022

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Abliva team is focused on delivering on goals and building value

HIGHLIGHTS

- December 2022:
  - Van Lanschot Kempen initiates coverage
  - Commencement of Phase 2 global, pivotal FALCON study on time
- January 2023:
  - Recruitment of VP, Clinical Operations
- February 2023:
  - Granting of NV354 patent in the U.S.
  - Proposal of new chairman of the Board, Edwin Moses

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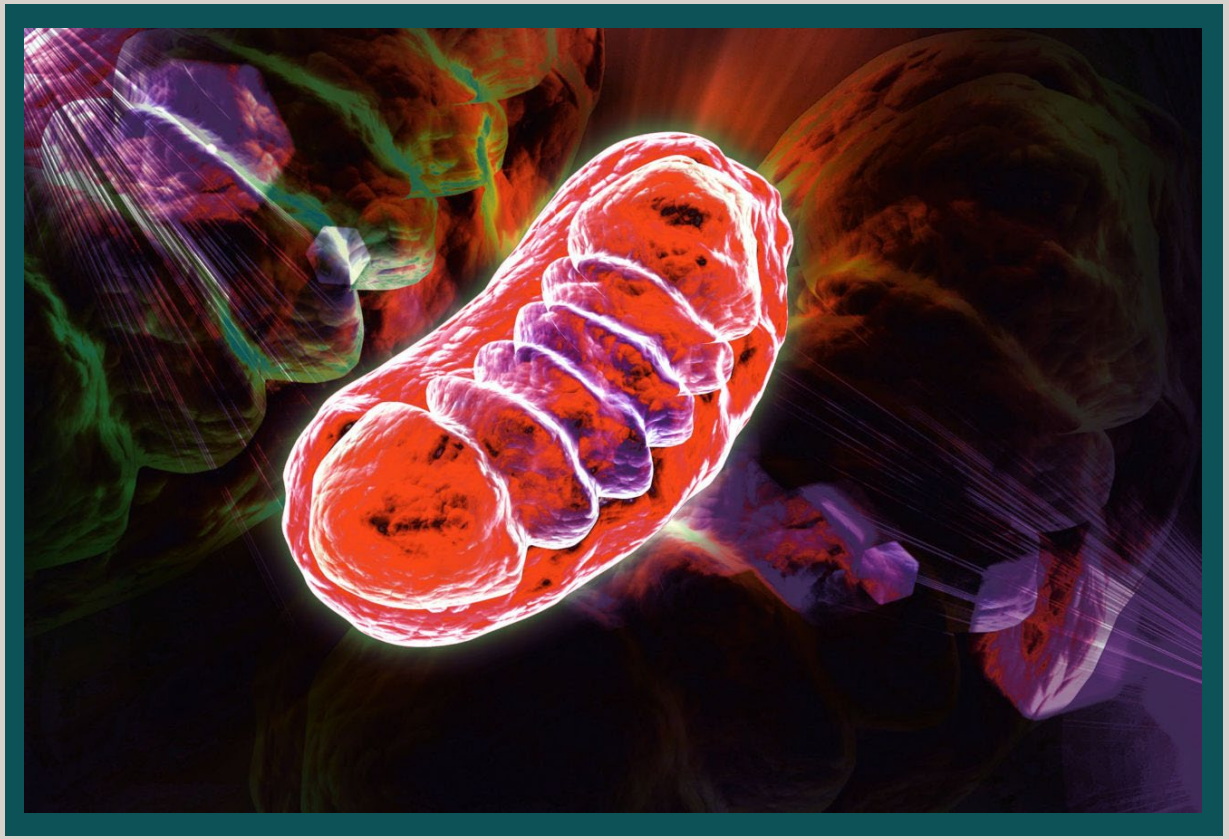
What are mitochondria?



- Powerhouse of the cell, they generate the chemical energy needed to power every cell in your body
- The number of mitochondria in the cell is dependent upon the amount of energy needed
  - Muscle
  - Liver
  - Brain
- Mitochondrial DNA comes from mom and is prone to mutations

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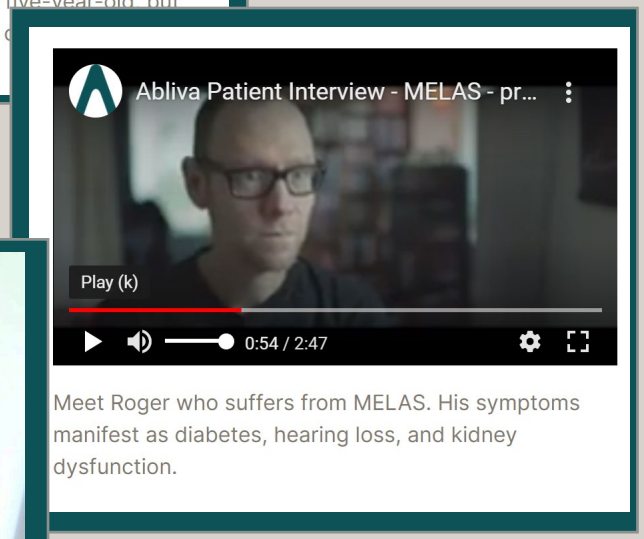

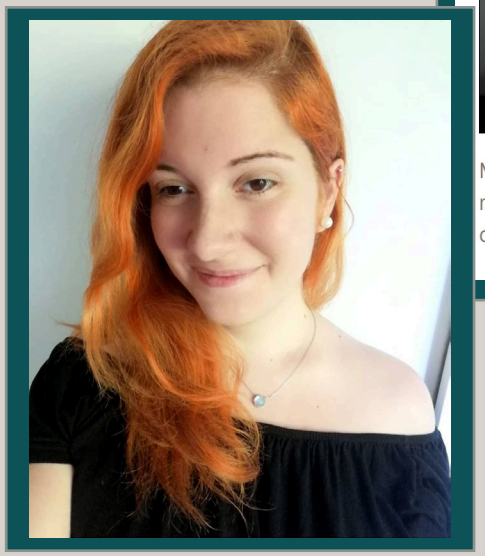

What are primary mitochondrial diseases?

- Rare diseases affecting 1:5000 individuals
- Devastating, debilitating disorders
- Life expectancy between 10 and 35 years of age
- No approved therapies for systemic disease
- Organs requiring lots of energy impacted most severely (muscles, brain, liver, etc)

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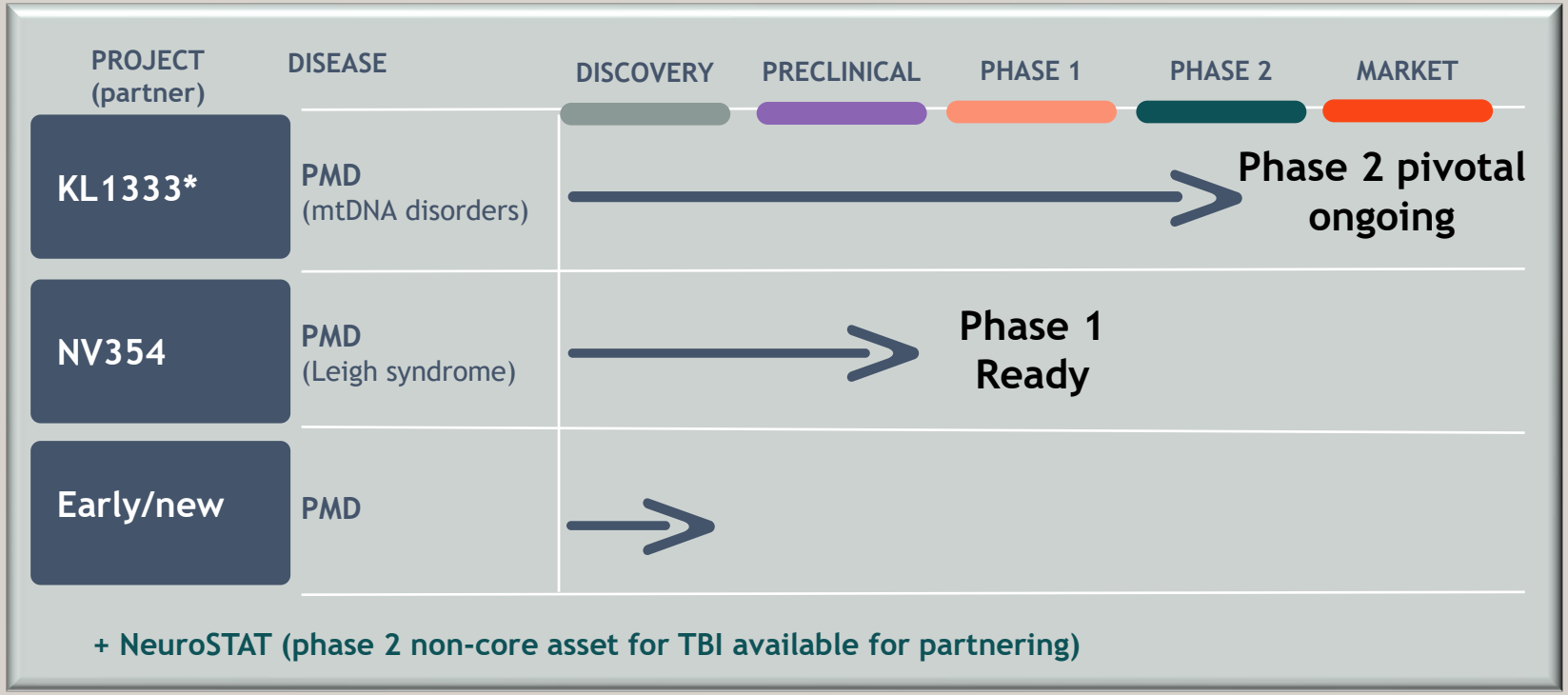
A portfolio of first-in-class therapies targeting underlying pathology in Primary Mitochondrial Diseases (PMD)



PMD= Primary Mitochondrial Disease, mtDNA disorders are disorders resulting from a mutation in the mitochondrial DNA.

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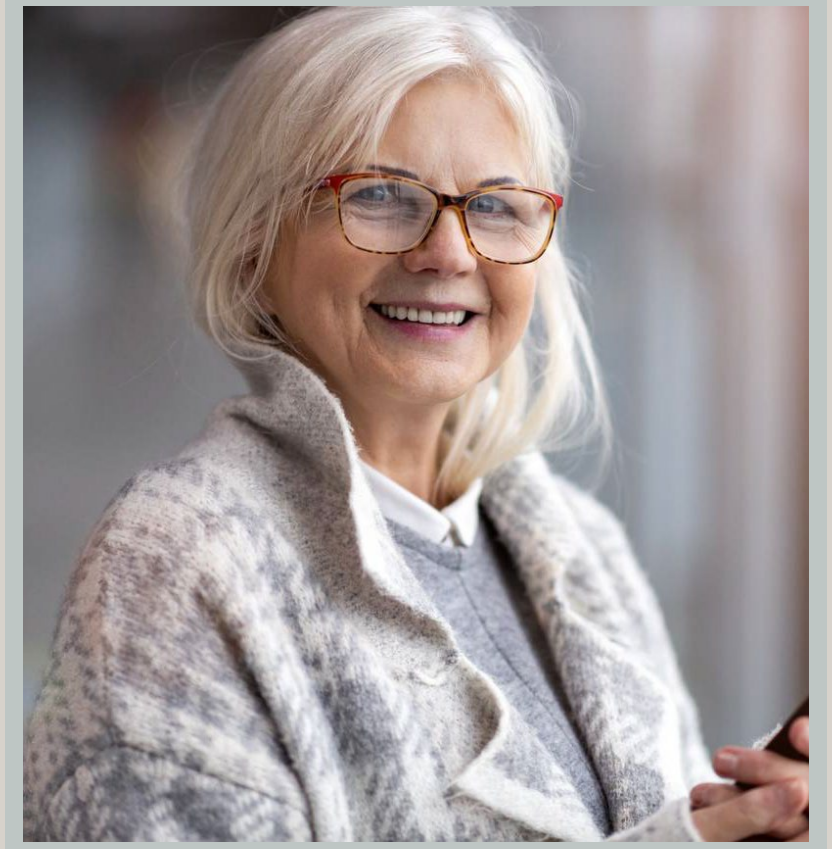
KL1333 is being developed for adults with PMD who suffer from fatigue and muscle weakness



- **Key Highlights:**
  - Modulator of NAD+/NADH levels as substrate of NQ01
  - Strong safety across multiple studies including:
    - Dosing in >100 healthy volunteers and patients
    - Drug-drug interaction study
    - Chronic toxicology studies
  - Signals of efficacy in placebo-controlled Phase 1b study in PMD patients
  - Phase 2/3 registrational study commenced December 2022 and will run first forty patients to an interim analysis
  - Large commercial opportunity with >$1bn blockbuster peak sales potential

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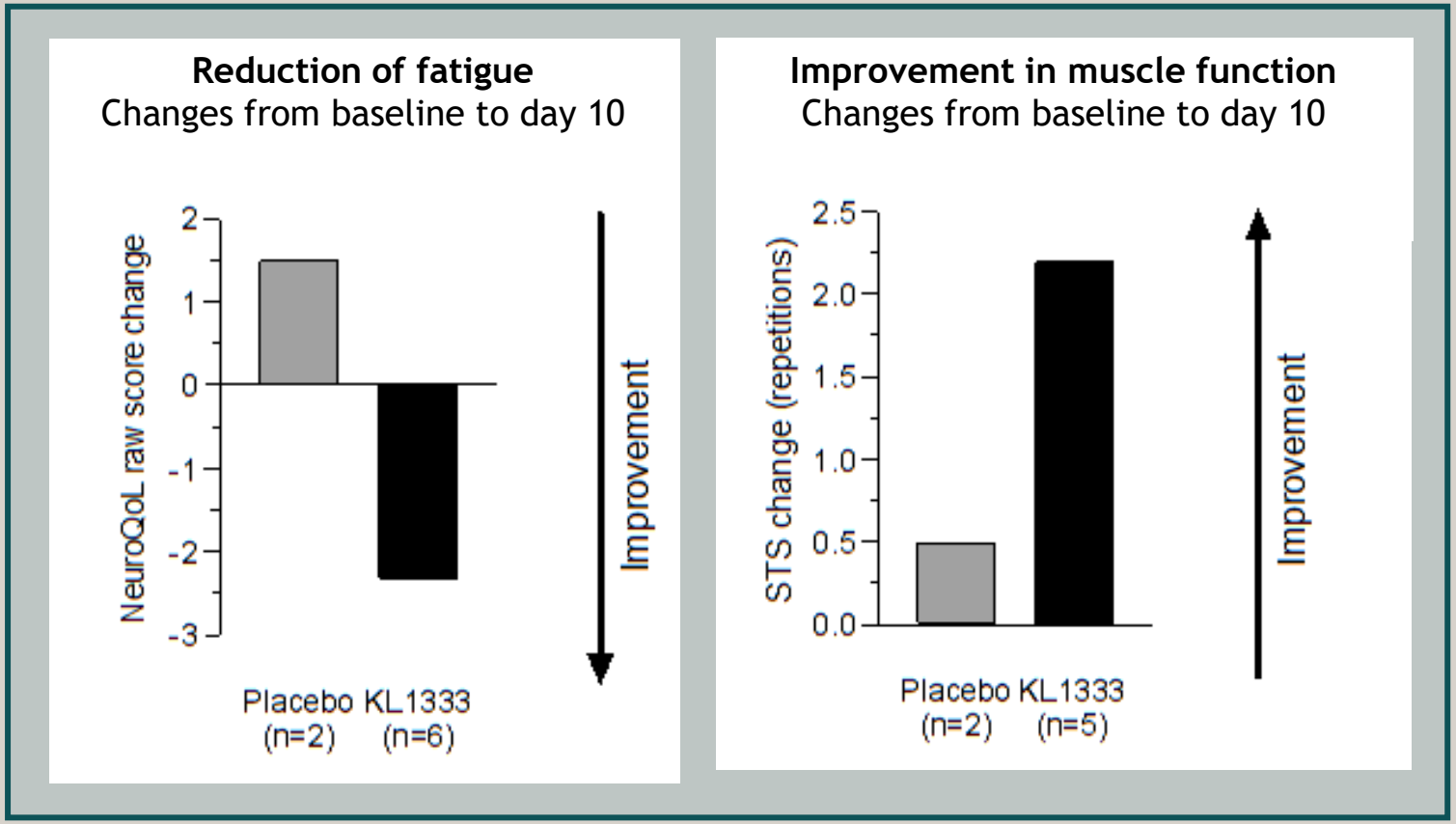
KL1333 showed signals of efficacy after 10 days, 50 mg/day in PMD patients



Phase 1b study data:

- Clinically meaningful effect signals on fatigue
- Clinically meaningful effect signals on muscle weakness and endurance
- Exposure / effect relationship
- Target engagement demonstrated

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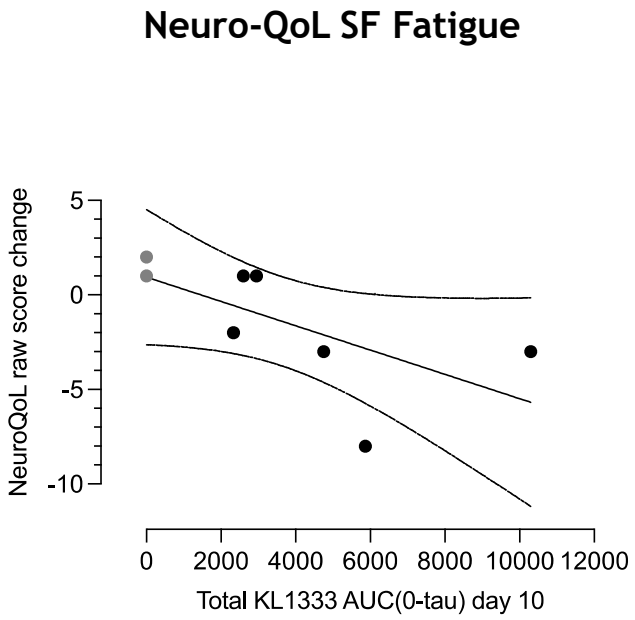
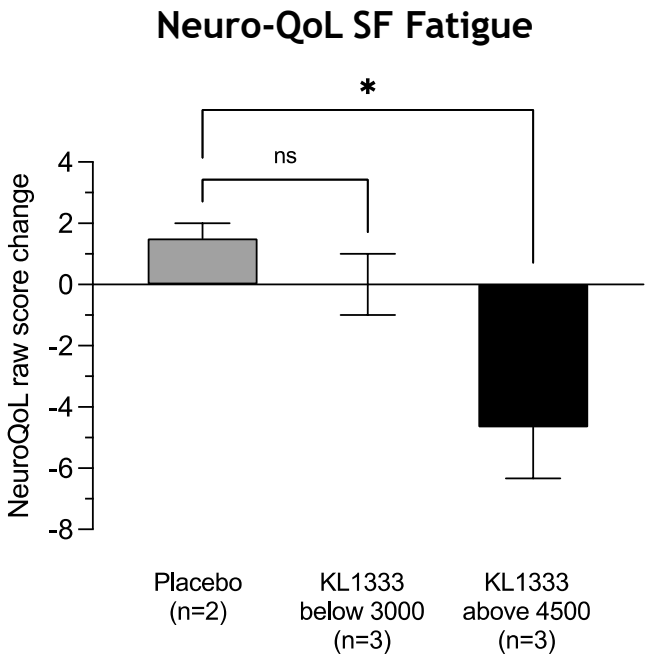
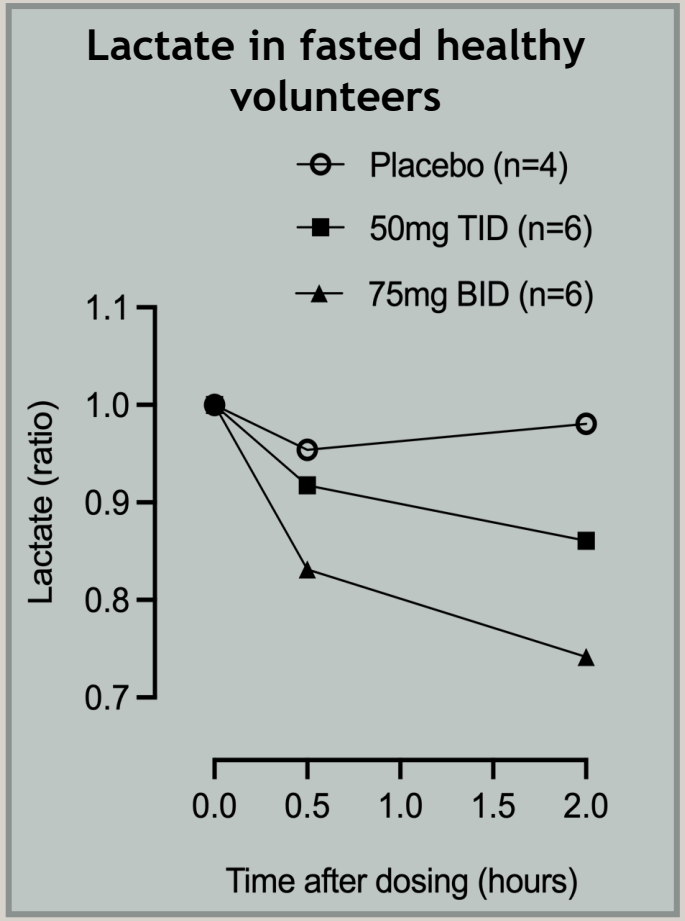
Correlation between exposure and efficacy exists for all three endpoints.

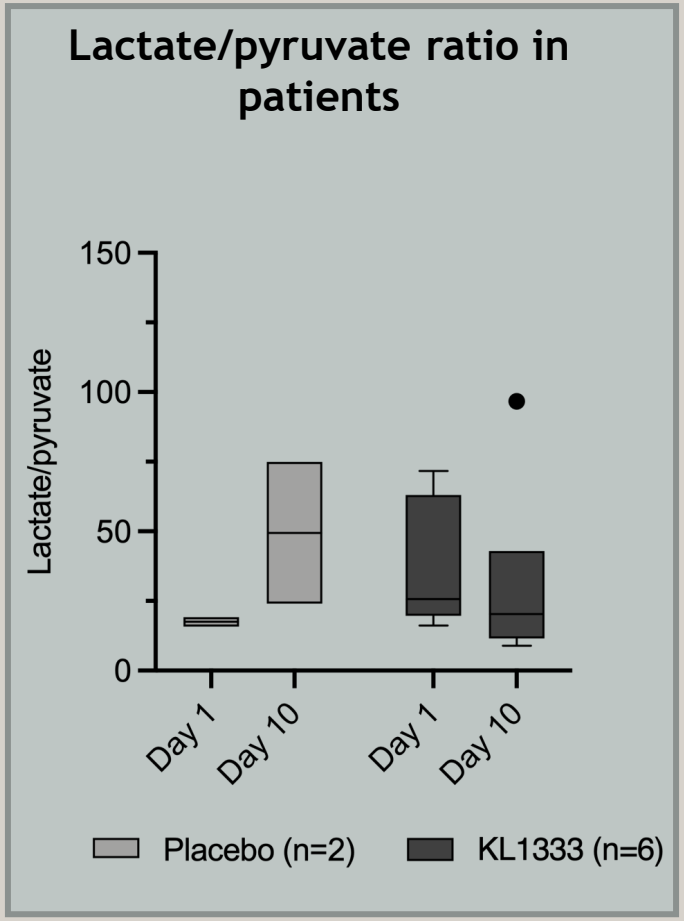
Reduction of fatigue in relation to exposure of KL1333





Target engagement was confirmed with biomarker data on lactate/pyruvate





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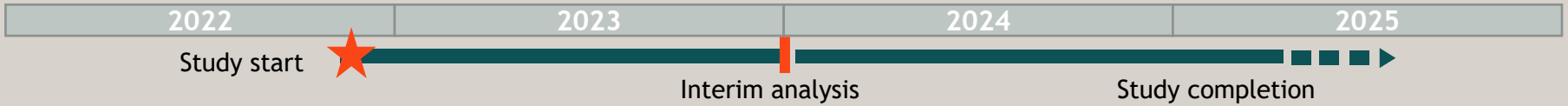
Global, registrational FALCON Phase 2 study commenced December 2022



- **Design:** Randomized, double-blind, parallel-group, placebo-controlled (40% placebo - 60% active)
- **Patients:** Adult Primary Mitochondrial Disease (PMD) patients with:
  - Multisystemic mitochondrial DNA-related disease (according to category 6* of the International Classification of Inborn Metabolic Disorders)
  - Disease expressions involving at least chronic fatigue and mitochondrial myopathy/exercise intolerance
- **Treatment:** Oral twice daily dosing (100mg/day) for 12 months (including dose titration phase)
- **Size:** Adaptive platform design of 120-180 patients financed to interim analysis
- **Endpoints:**
  - Alternate Primary: Fatigue (validated for PMD), 30 Second Sit-to-Stand
  - Secondary: Clinician- and Patient- Global Impression of Disease Severity; NMDAS**; patient-specific activity assessments

*Including MIDD-MELAS-m.3243A>G associated spectrum disease, single large scale mtDNA deletion associated KSS-CPEO spectrum disorders, MERRF, and other multisystemic mitochondrial DNA-related disease.; ** Newcastle Mitochondrial Disease Adult Scale (NMDAS) (Schaefer et al., 2006)

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Few competitors exist; race is on for the lead

Peripheral Therapies

Reneo PPARδ modulator; PMM	Start: 2H21 Readout: 2H23
Astellas PPARδ modulator; PMM	Start: 2H21 Readout: Unknown
ABLIVA	Start: 2H22 IA: 4Q23/1Q24
Stealth MOA unknown; nDNA PMD	Start: 2H22 Readout: Unknown

Preclinical	Phase 1	Phase 2	Phase 3	Approved

CNS Therapies

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Cyclerion
sGC stimulator; MELAS

Khondrion
Oxidative stress mod; MELAS

PTC
Oxidative stress mod; PMD epilepsies

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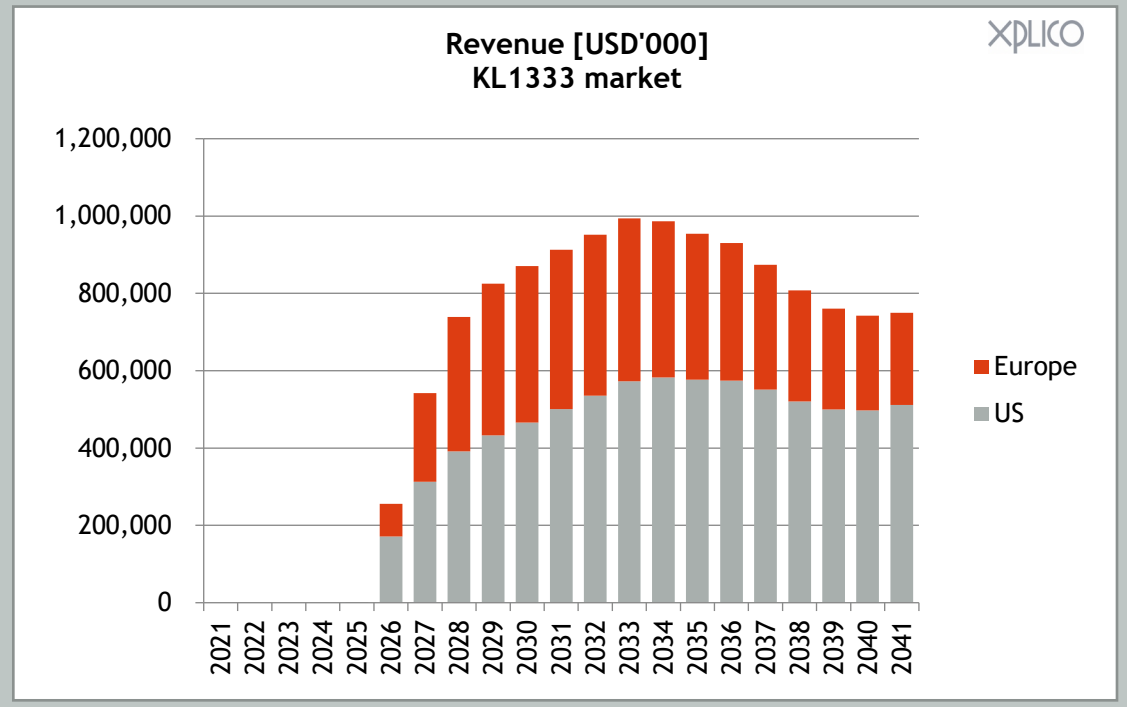
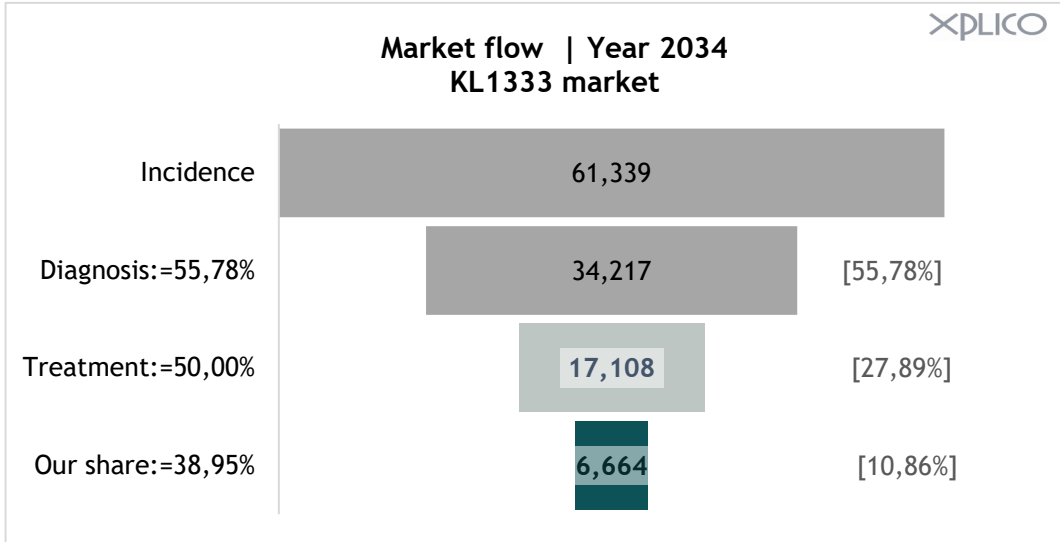
Even with conservative assumptions, KL1333 has blockbuster potential

Base case TPP: Peak sales $1.0B in US, EU



Assumptions: KL1333 treated population on the right; US price of $160K/year (EU $80K)

PMD population & KL1333 market



NV354 is a brain-penetrable prodrug of succinate

- Designed by Abliva scientists
- Energy replacement therapy aims to modify disease progression
  - Pediatric Leigh syndrome is target
    - Severe multiorgan deterioration
    - Life expectancy <five years
    - Rare disease (25:1,000,000 live births)
  - Expansion to other PMDs, other CNS
- Asset now ‘clinic ready’ following UK regulatory (MHRA) scientific advice

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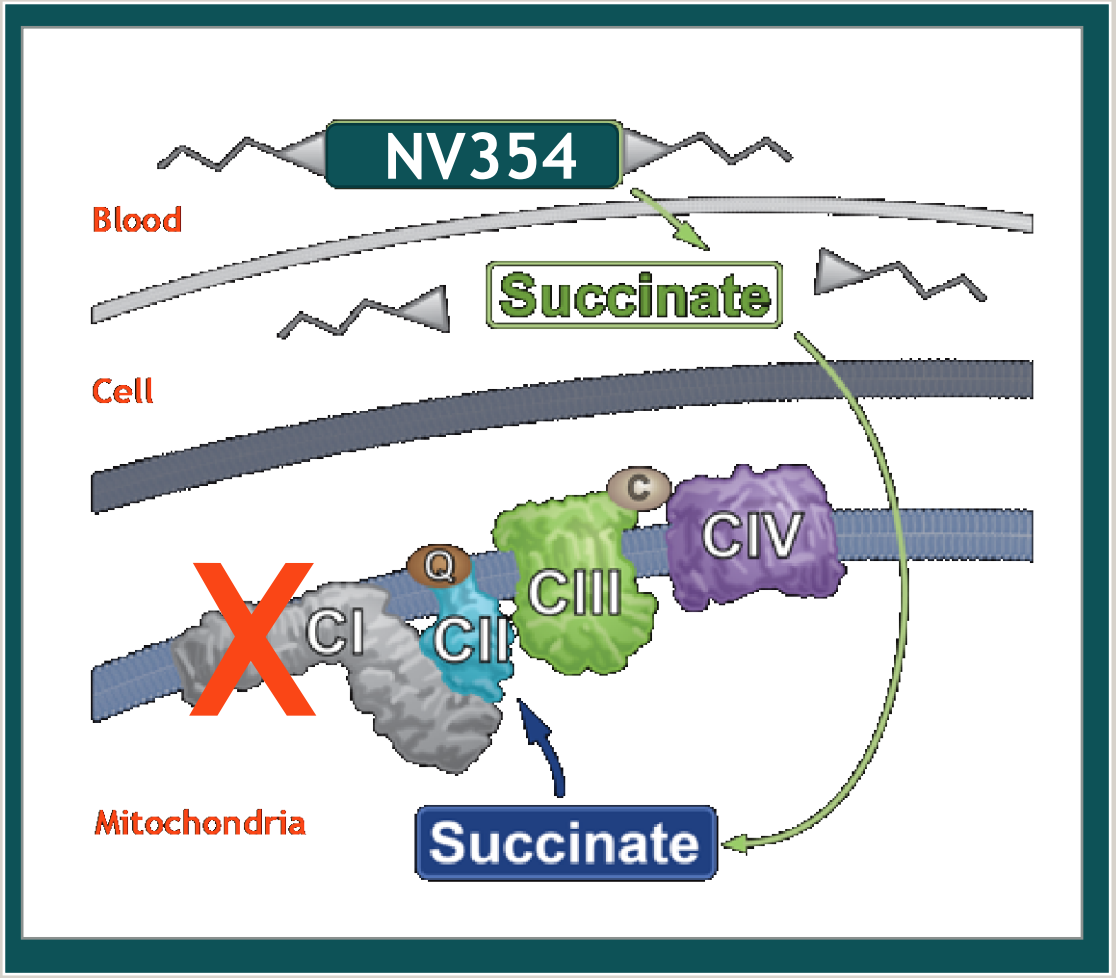
NV354 bypasses defective Complex I in ETC*

- Complex I dysfunction is one of the most common causes of mitochondrial disease
- Disease modifying potential
  - Protects mitochondria and loss of organ function
  - Prevents complications caused by acute energy crisis
- Succinate enters the cell through innovative pro-drug approach



*ETC: Electron Transport Chain

Ehinger et al. Nature Communications, 7:12317, August 2016

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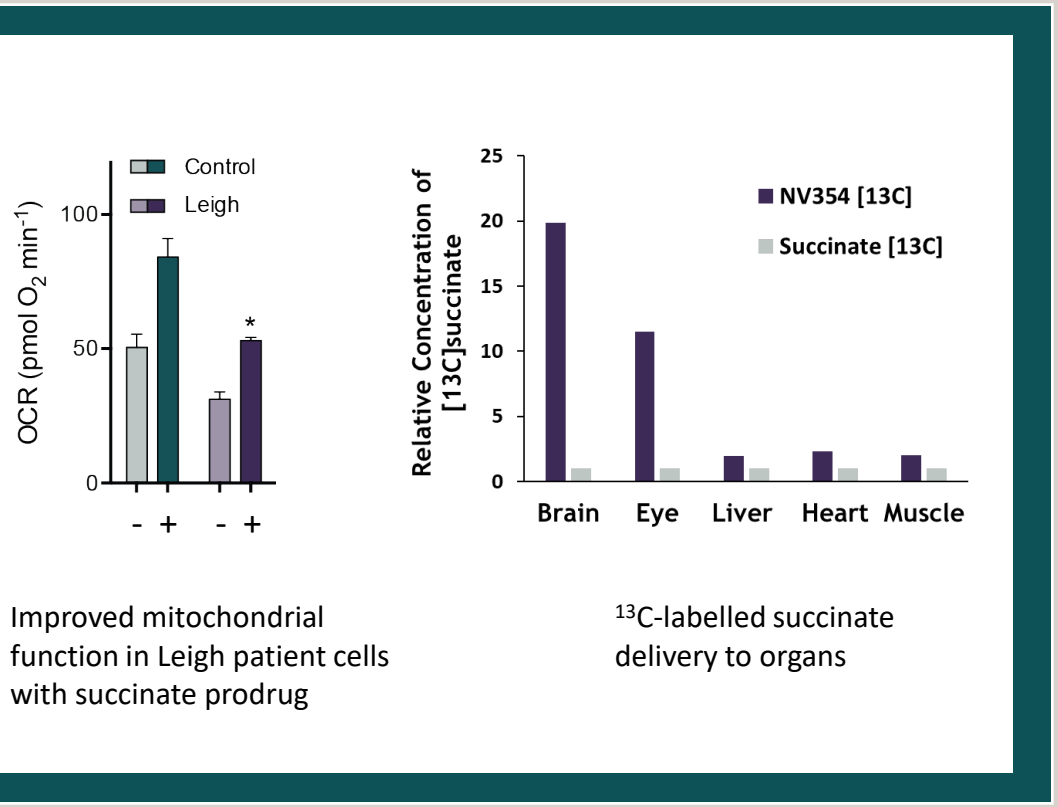
NV354 corrects underlying biochemical dysfunction in Leigh patient cells



Improved mitochondrial function in Leigh patient cells with succinate prodrug

^{13}C-labelled succinate delivery to organs

- Good 'drug properties'
  - High oral bioavailability
  - High brain distribution
  - Good tolerability in toxicology

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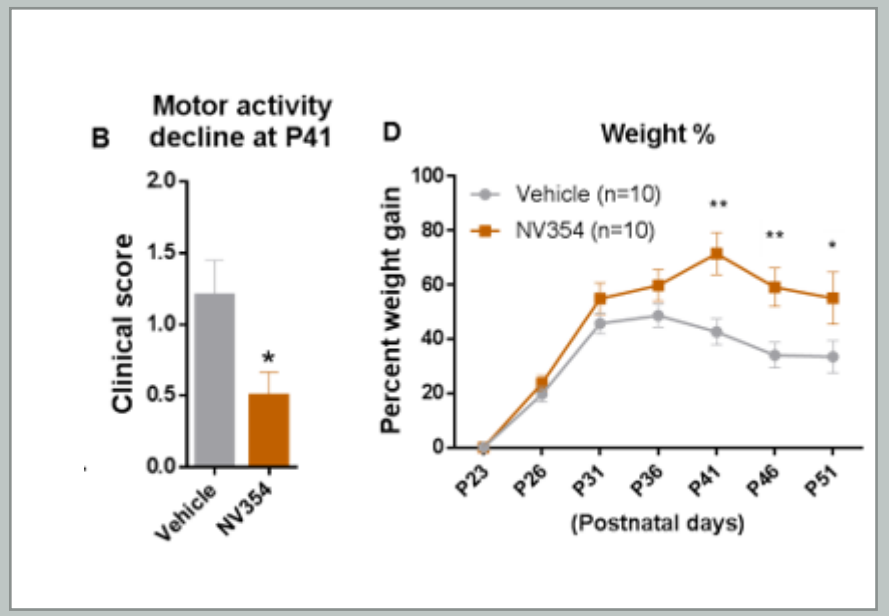
Leigh disease model study suggests impact on brain inflammation, motor activity and weight

Ndufs4 model with complex I dysfunction



- Decrease in neuroinflammation
- Delay in the presence of clinical/motor signs
- Improved weight gain

Unpublished data; Quintana lab, UAB (Barcelona)

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The team at Abliva brings wealth of experience in drug development and mitochondrial medicine

Ellen Donnelly, PhD
CEO

- Formerly CEO of Modus Therapeutics (SCD focus)
- Leadership positions at Pfizer
- Corporate consulting, SVB Leerink
- PhD, Yale School of Medicine

Catharina Johannson
CFO

- Abliva CFO since 2013
- Senior financial positions in companies such as Assa Abloy, Entrematics, Bong, Alfa Laval Europe
- Interim CFO Cellavision

Magnus Hansson, MD/PhD
CMO

- Abliva since 2008; CMO since 2016
- Consultant physician at Skåne University Hospital and Associate Professor at Lund University with long experience in mitochondrial medicine development

Eskil Elmer, MD/PhD
CSO

- Co-Founder, Abliva
- Professor, Lund University
- 20+ years of research in mitochondrial medicine

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The Abliva team is supported by a strong Board and Scientific Advisory Board (SAB)

Board of Directors

- **David Laskow-Pooley**
  Director of the Board of Marker Therapeutics Inc. (England), Pharmafor Ltd, England, and LREsystem Ltd, (England).
- **David Bejker**
  Affibody Medical AB (CEO, Board), LIDDS AB (Board), Amylonix AB (Board).
- **Roger Franklin**
  Partner, Hadean Ventures
- **Denise Goode**
  QED Life Sciences Ltd (CEO, Board). 20 years with AstraZeneca in senior finance & business roles.
- **Jan Tornell**
  Innoext AB (CEO, Board), LIDDS AB (Chair), and Glactone Pharma AB (Chair), Diaprost AB (Board), LIDDS Pharma AB (Deputy Board).

Scientific Advisory Board

- **Amel Karaa, MD,PhD,** Ass. Prof, internist and medical geneticist, Dir. Mito Disease Program, Harvard, MGH. President of the Mitochondrial Medicine society.
- **Bruce Cohen, MD, PhD,** Paediatric Neurologist, Director, Neuro-Developmental Science Center, Akron
- **Michio Hirano, BA, MD, PhD,** neurologist, Columbia. Co-director the North American Mitochondrial Disease Consortium
- **Marni Falk, BS Sci, MD, PhD**, Clinical Geneticist, Associate Prof. of Pediatrics at Penn, CHOP. Advisory Board of UMDF, founding member of the CHOP Center for Mitochondrial and Epigenomic Medicine
- **Grainne Gorman, MD, PhD,** Newcastle, Neurologist, co-founder of the Wellcome Centre for Mitochondrial Research Leader of the Newcastle Mito Hub
- **Robert Pitceathly, MD, PhD,** Neurologist, Clinical Scientist, Department of Neuromuscular Diseases UCL Queen Square Institute of Neurology
- **Michelangelo Mancuso, MD, PhD,** Ass. Prof, Neurologist, Coordinator Clinical Neurogenetics and Rare Diseases, University of Pisa, Italy

Company completed a new financing round in 2Q22

- SEK 200M raised through a directed share issue (150M) and a fully underwritten rights issue (50M)
  - Raise happened in a market where biotech sector is lowest level for 20 years; ECM transactions in life sciences are down 95% compared to 2021
  - Top 10 biotech deal done in EU in 2Q according to BioWorld
  - Deal was done at a 7% discount (versus current average of 35-40%)
- Attracted new, high-quality life science and institutional investors
- Rights issue provided current shareholders with the opportunity to participate

Company is fully financed to a key milestone for lead asset and has cash runway for 24 months

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Experienced team with 20+ years in mitochondrial research and drug development

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Full **R&D capabilities** with a plan to **build to commercialize lead asset**

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- 24 months of runway with raise in June 2022

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