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

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Investment Highlights

Novel therapy targets underlying pathology of mitochondrial disease

- Novel therapeutic approach **modulates underlying pathophysiology** to promote mitochondrial biogenesis and restore energy metabolism
- Supports symptom reduction and disease modification in primary mitochondrial disease

Attractive commercial opportunity addresses high unmet need

- No approved medicines for systemic PMD
- **Opportunity for >1B+ US, EU peak sales**
- Lead program KL1333 is poised to be the first to market treatment for mitochondrial disease

Pivotal FALCON trial of KL-1333 ongoing with well-defined path forward

- **Positive interim analysis confirms safety and two 'shots on goal' strategy**
- Phase 2 optimized for success, incorporates patient input and builds on strong efficacy and favorable safety data
- KL1333 poised for NDA submission following FALCON completion

First-in-class assets with strong regulatory and IP packages

- **KL1333: Fast Track Designation in US, Orphan Drug Designation in US and EU**
- NV354: Phase 1 ready in neurological diseases; Orphan Drug Designation in the US, EU
- Additional discovery programs in development with opportunities to drive long-term value

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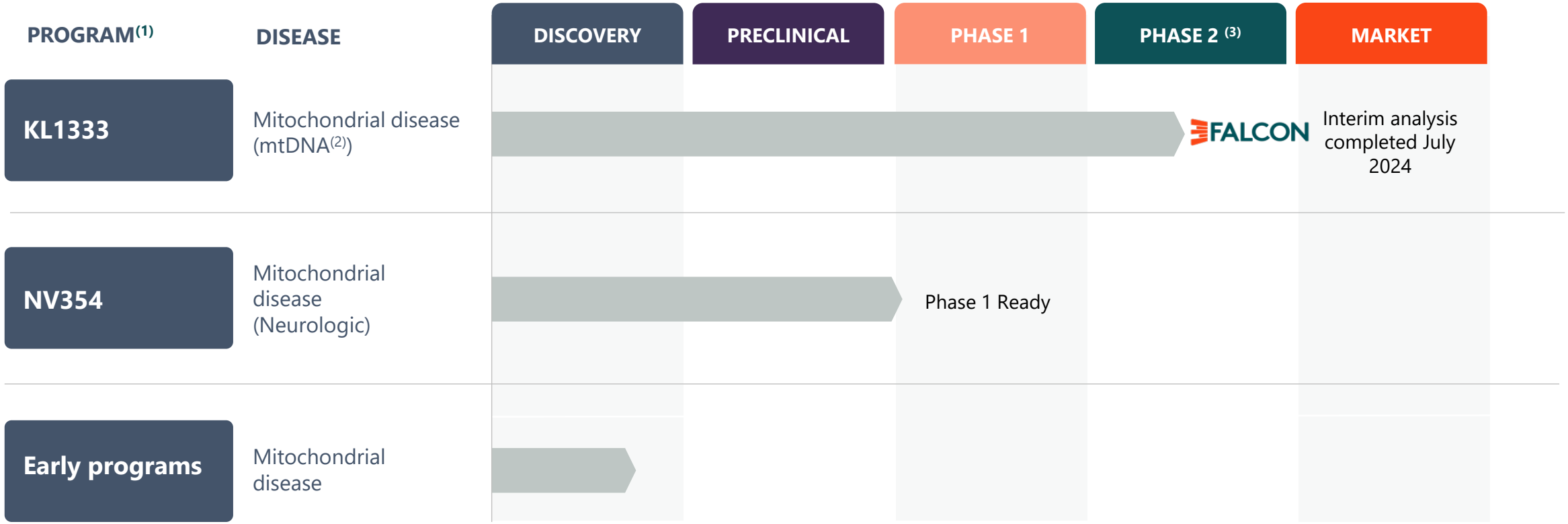
Abliva is focused on becoming a global leader in mitochondrial medicine

Experienced team with 30+ 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**

Publicly traded on NASDAQ Stockholm (ABLI)

A portfolio of first-in-class therapies target underlying pathology in Primary Mitochondrial Disease



(1) KL1333 has Orphan drug designation in the US and Europe. Fast track designation in the US.; NV354 has Orphan drug designation in the US and EU
(2) mtDNA-related mitochondrial disease caused by mutation(s) in mitochondrial DNA (as opposed to nuclear DNA).
(3) Given that mitochondrial disease is an orphan indication, a Phase 2 study in these patients, if successful, can have the potential for market approval.

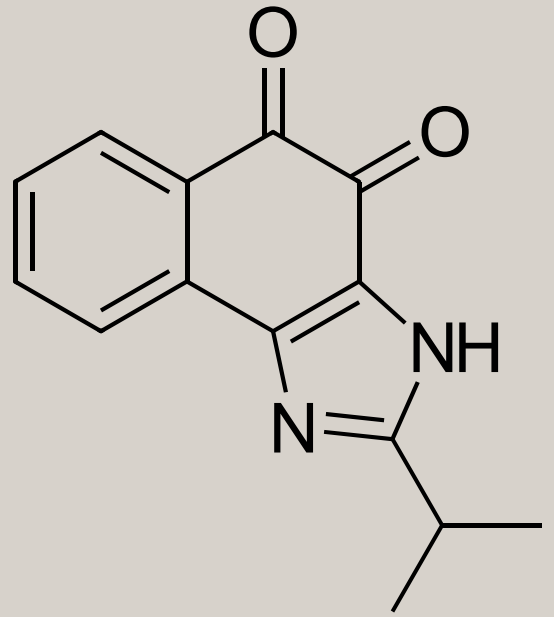
The portfolio is protected and expedited with a strong regulatory and IP package

KL1333 (orally available NAD⁺ /NADH modulation)

- Orphan Drug Designation in US, EU
- Fast Track designation in US
- Composition of matter patent until at least 2034
- Expansion of protection ongoing

NV354 (orally available, brain-penetrant succinate prodrug)

- Orphan Drug Designation in US, EU
- Three patent families protect the asset until at least 2035
- Expansion ongoing; US patent received in Feb 2023 protecting isolated forms of NV354



KL1333 structure

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Roadmap

2023 - 2024 Key Accomplishments

2023

- NV354 patent granted in the U.S. in February
- NV354 received Orphan Drug Designation in the U.S. in April
- First patient dosed in KL1333 FALCON study achieved in June
- KL1333 received Fast Track Designation in September
- NeuroSTAT® for Traumatic Brain Injury outlicensed to Owl Therapeutics in November
- NV354 received Orphan Drug Designation in the E.U. in December
- All patients recruited into Wave 1 of the FALCON study in December

2024

- Financing completed to elongate runway around IA
- FALCON Wave 1 patients complete 24 weeks of dosing
- FALCON interim analysis completed

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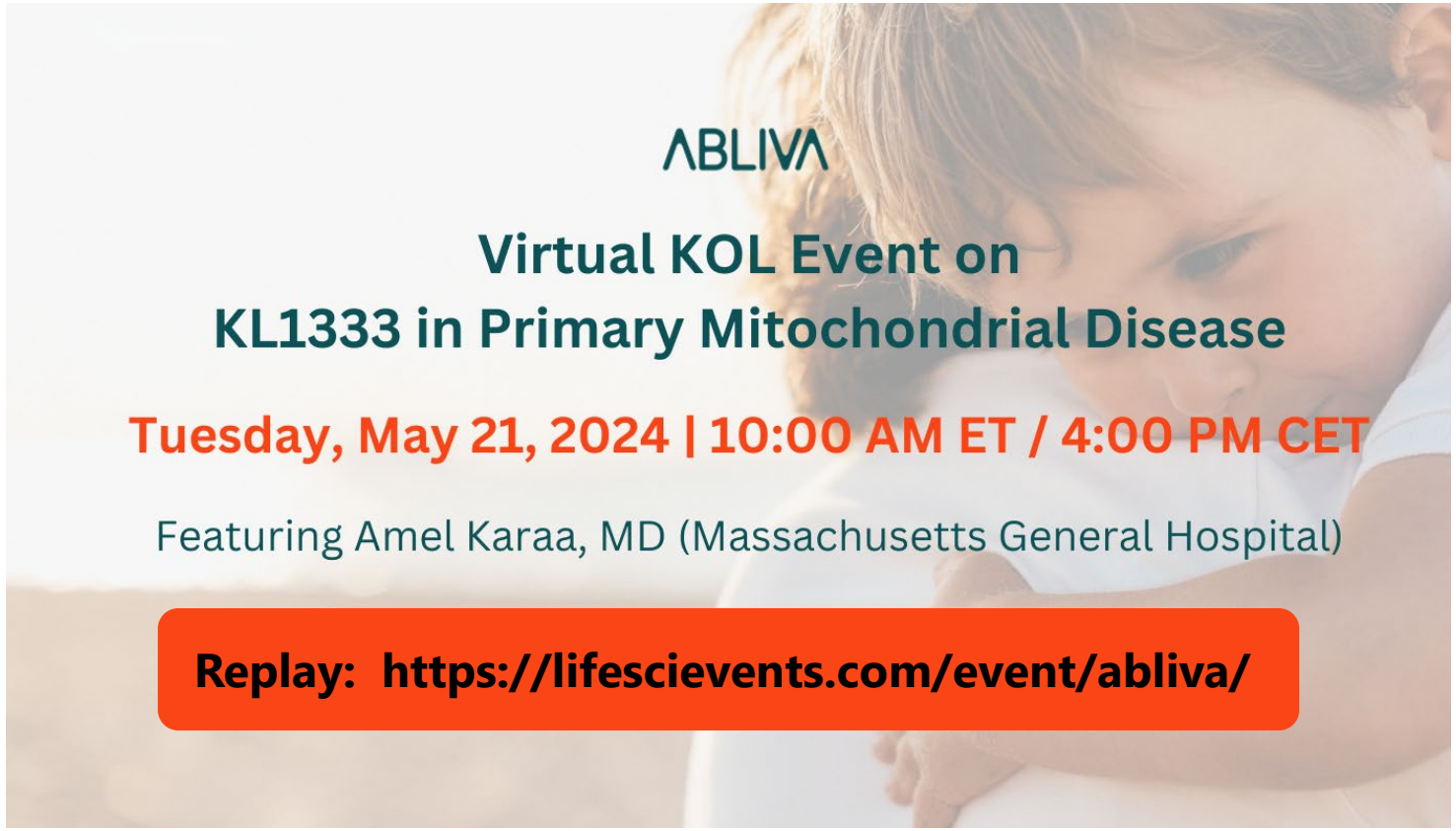
KOL Event: May

KOL R&D event was a highlight of recent investor events.



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Opportunity in Primary Mitochondrial Disease

First-in-Class Rare Disease Therapy with Blockbuster Peak Sales Potential

1. Positioned to become **first Standard of Care** in **Primary Mitochondrial** diseases
2. **Large commercial opportunity** with **~100,000 patients** in US / EU
3. **Premium** rare disease **pricing** with preliminary **payer work** suggesting net price of **$190k - $265k** in US
4. **Concentrated centres of excellence** and **advocacy groups** leading to **efficient** rare disease **commercial infrastructure (~20 reps needed in US)**
5. Small molecule pill has low COGS and **easy** route of **administration**

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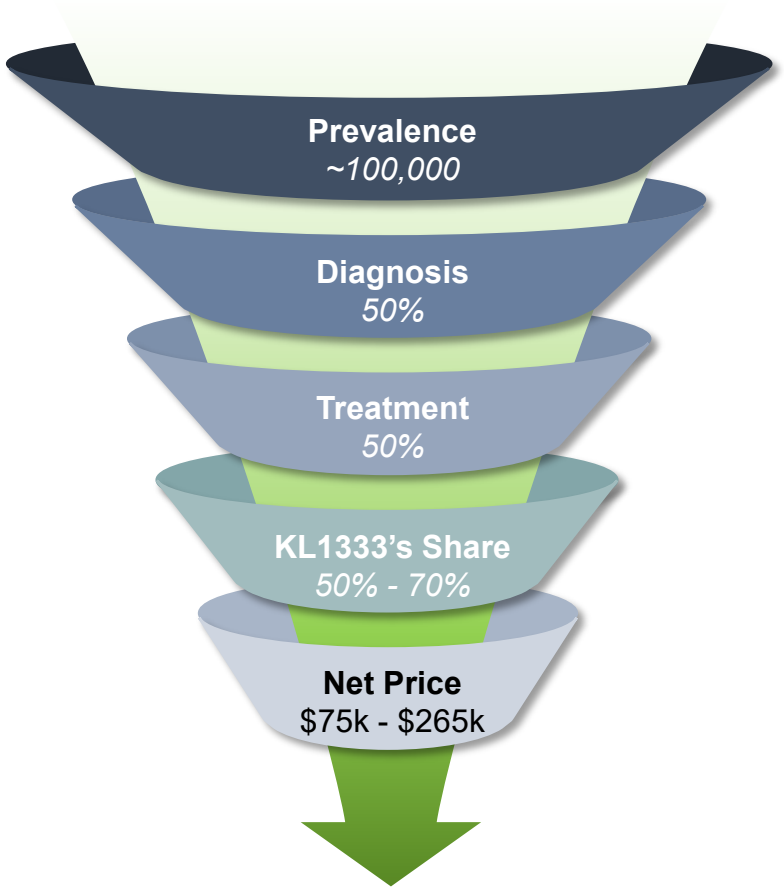
Even with Conservative Assumptions, KL1333 has Blockbuster Potential



Market Build at Peak Year

Conservative			Base	
U.S.	Europe		U.S.	Europe
54,000	50,000	Prevalence[1]	54,000	50,000
27,000	25,000	Diagnosed Patients	27,000	25,000
13,500	12,500	Treated patients	13,500	12,500
			Changes from Conservative Case	
6,750	6,250	KL1333 patients	9,500	8,750
$190k	$75k	Net Price	$265k	$130k

Preliminary assumptions suggest peak sales opportunity of $1bn - $4bn

Source: Broker research, Company Information
1. Through US and Europe

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Analogs and Public Equity Research Support the Large Commercial Opportunity, Especially with Key Competitors out of the Way

Key Themes of Commercial Opportunity – Street view

MARKET WHITESPACE
No currently approved treatments for mitochondrial disease creating an opportunity for rapid and widespread adoption of a new drug

LIMITED COMPETITION
Limited potential competition given early-stage phase of competing programs

LARGE TAM
Despite lower market penetration rate (25-35% due to diagnostic challenges), very substantial global market of c.500k[1] patients to address

PRICE SETTING OPPORTUNITY
Reflecting orphan disease status and potential first-in-class therapy becoming new Standard of Care

Analogs Street View

> *"Despite poor diagnosis rates (about 47% according to literature), we model more than 15,000 confirmed PMM patients residing within the U.S. alone with no access to an FDA-approved therapy. We see **peak U.S. sales** of mavodelpar at **$1.48 billion** and in **EU territories** at **$1.12 billion** using 35% peak penetration rate."*
>
> Reneo — William Blair 14 Nov 23

> *"[…] in the US/EU we project **peak sales** of ~ **$910m/$530m**, respectively. Currently there is no approved treatment for PMM."*
>
> Reneo — Jefferies 21 Dec 21

> *"Launch in the U.S. and EU Is assumed to occur in 2026, with **peak revenue** of **$2.2bn** achieved in 2035."*
>
> Reneo — PIPER|SANDLER 04 May 21

> *"Reneo's first indication for EN001, which is PMM, is the most prevalent of the three indications, with an **estimated 504.1k patients globally**. We believe that REN001 has the potential to become a disease-modifying therapeutic that can **address the significant unmet need and capture the vast majority of these patients**."*
>
> Reneo — PIPER|SANDLER 04 May 21

Source: Broker research, Company Information
1. 66K patients in the US, 83K patients in EU, 288K patients in China, 25K in Japan and 43K patients in Brazil

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The Potential Benchmark Price Corridor for KL1333 in the EU5 is ~$90-225,000 and in the US it is between $140-350,000 Per Year

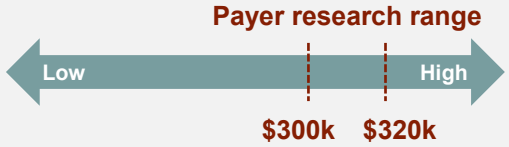
EU5 price benchmarks

$90,000 / yr
Yondelis (Sarcoma)
Zavesca (Gaucher)

$225,000 / yr
Galafold Fabrazyme (Fabry disease)

- Gaucher, Fabry diseases and rare oncology products commonly cited as benchmarks for PMD and KL1333 by EU Payers
- Remarkably, no links to cheaper products were made
- While the unmet need is relatively very high for oncology indications, payers reasoned that such products indicate the WTP for orphan products with low added benefit

USA price benchmarks



$140,000 / yr
Hetlioz (sleep disorder)
Wakix (narcolepsy)

$350,000 / yr
Galafold (Fabry disease)
Cerdega (Gaucher)

- The lowest price benchmark offered by US Payers were the narcolepsy orphan products
- Payers note that WTP will be higher for products that are perceived as disease modifying

> *"In terms of system features, I would say **something like Fabry or Gaucher disease is very similar in terms of some of the things that you have here**…"*
>
> ***UK Player***

> *"Italy is a budget impact archetype. […] **AIFA is likely to establish the price of product X based on an analogue in another indication, with the same epidemiology**. Roughly, not specific or not exactly the same number of patients, but in the same range…"*
>
> ***IT Player***

> *"… so if we're **willing to treat for excess daytime sleepiness** (Hetlioz), which is not the same as fatigue but similar in that it is something experienced and not due to an organ dysfunction..."*
>
> ***US Player***

Source: Company information

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Thin competitive landscape with clear path to first-to-market opportunity

Asset	Type	MoA	Stage *RoA*	Patient Group Focus	Commentary
ABLIVA **KL1333**	Small Organic Molecule	NAD^+/NADH modulation	Pivotal *Oral*	mtDNA mutations (mtDNA deletion, m.8344A>G, MELAS-MIDD)	• Ongoing potentially registrational Ph. 2 trial evaluating alternative independent primary endpoints of fatigue and myopathy • Reported 24w interim analysis and validated both endpoints (reduction in fatigue and improvement in muscle function), evaluating 40 patients across 6 countries (18 sites) and informing size of Wave 2 • Favourable recommendation by the DMC to continue the FALCON study with a total of 180 patients, validating the overall study design and confirming the strong safety profile of KL1333
Stealth BIOTHERAPEUTICS **Elamipretide**	Tetrapeptide	Mitochondrial cardiolipin-binding peptide	Pivotal *Subcutaneous*	Subgroup of mitochondrial myopathy patients (nDNA mutations)	• After several trials, focus of ongoing phase 3 trial is on a subgroup of mitochondrial myopathy patients (nuclear DNA mutations), which represents a smaller subset vs. mitochondrial DNA mutations; Phase 3 expected to readout in 2024 • Currently in discussions with FDA re: open-label data for ultra rare Barth syndrome (150 patients globally)
Tisento THERAPEUTICS **Zagociguat**	Small Molecule	Guanylate cyclase (sGC) stimulator (amplifies NO signaling)	Phase IIb ready *Oral*	MELAS	• Open-label MELAS phase 2a completed. Phase 2b trial planned with focus on fatigue, myopathy and cognition
KHONDRION **Sonlicromanol**	ROS-redox modulator	Oxidative stress modulator	Phase IIb completed *Oral*	MELAS (pediatric)	• Focus on m.3243A>G patients (primarily MELAS). Smaller Phase 2a and 2b placebo-controlled studies have been completed (missed primary endpoint) with some promising data seen in post-hoc analyses. Phase 3 program in planning process

Remaining high unmet need for differentiated therapeutic options: clinical stage landscape behind Abliva, with complementary approaches to KL1333 (nDNA subset, pediatric, etc.)

Source: Broker Research, Company information
1. GA = Geographic Atrophy; 6MWT = 6-minute walking test

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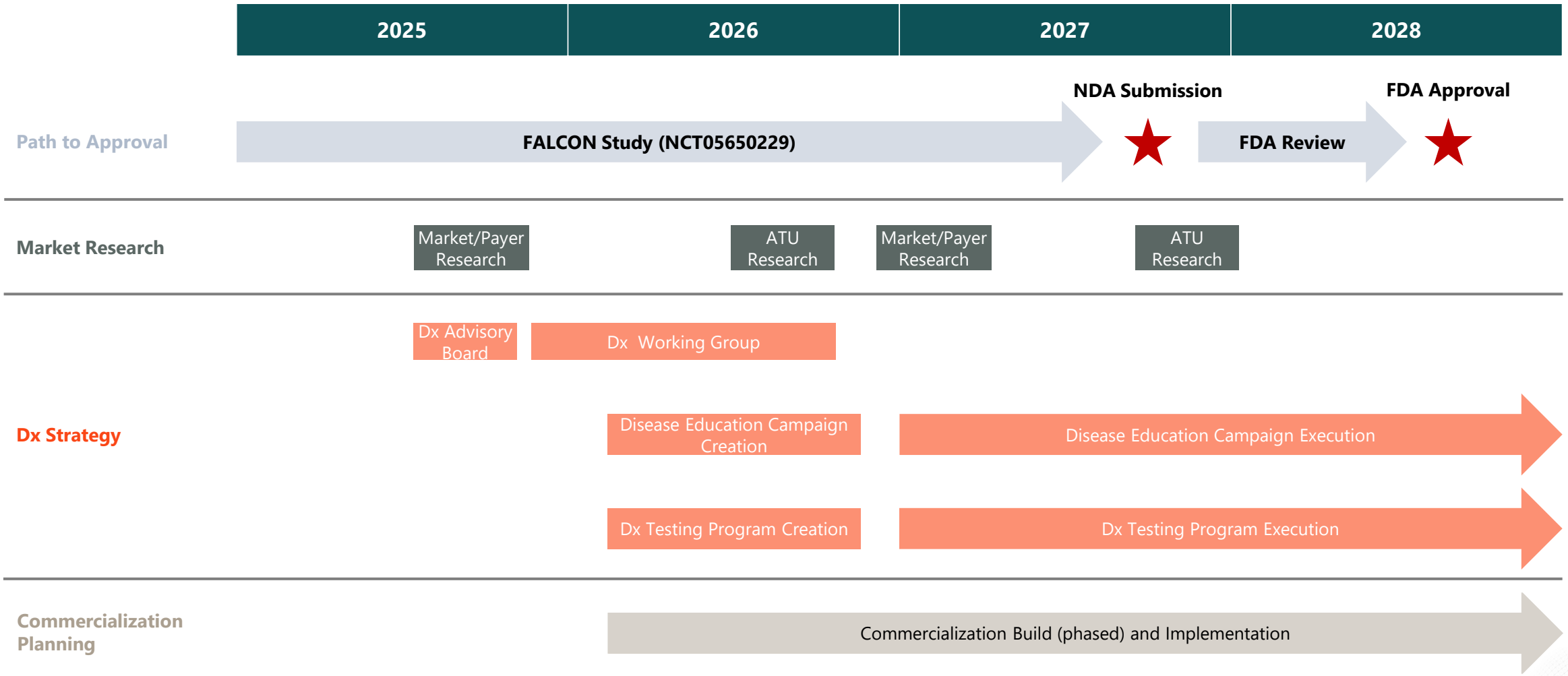
Market Development: Key Initiatives



Note: ATU: Awareness, Trial and Usage, Dx: Diagnostic

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KL1333

What is primary mitochondrial disease?

What are mitochondria?

'Powerhouse' of the cell, they generate the chemical energy needed to power the cells of your body

- Energy is produced in the form of ATP via cellular metabolism which relies on numerous NAD^+-dependent reactions and the electron transport chain (ETC)
- NADH is oxidized to NAD^+. A high NAD^+/NADH ratio indicates a high potential for ATP production; the ratio is vital for mitochondrial function and overall cellular health
- Maintaining an optimal NAD^+/NADH ratio is therefore essential for proper functioning and survival of cells

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The number of mitochondria in the cell is dependent upon the amount of energy needed: muscle, liver, brain

Mitochondrial DNA is maternally derived and is prone to mutations

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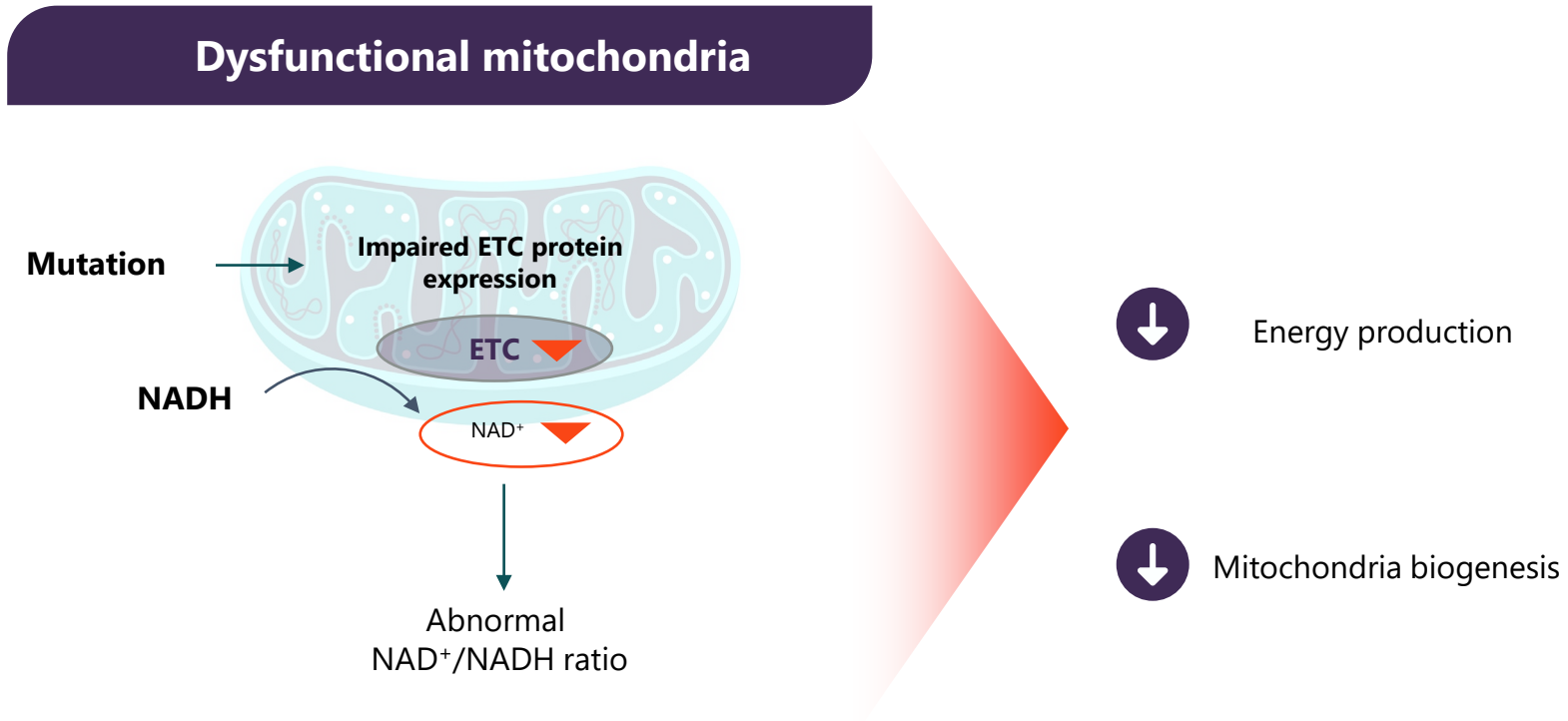
Dysfunctional mitochondria have a disrupted ratio of NAD⁺/NADH



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

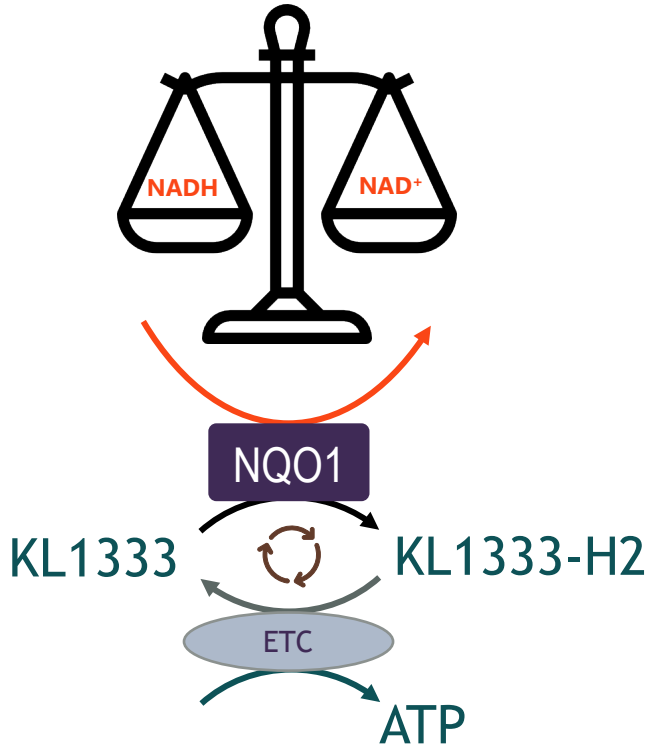
NAD= Nicotinamide adenine dinucleotide, NADH= Nicotinamide adenine dinucleotide + hydrogen, ETC=Electron Transport Chain

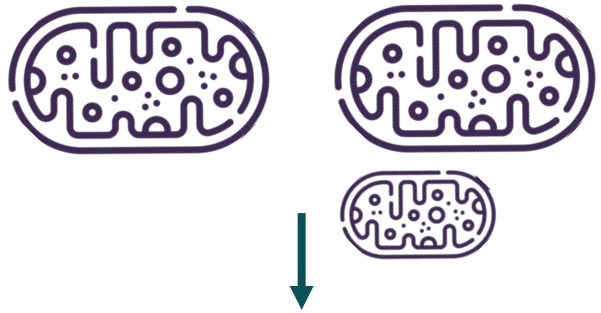
The ratio is a key indicator of the cell's redox state:

- A disrupted redox balance can lead to reductive stress, which disrupts cellular energy metabolism and contributes to organ dysfunction and disease deterioration

KL1333 corrects underlying pathophysiology of mitochondrial disease

Normalized NAD⁺/NADH ratio





1. Restored energy regulation and improved ETC function
2. Mito biogenesis stimulated

Overall results:

Symptom reduction, disease modification

NAD= Nicotinamide adenine dinucleotide, NADH= Nicotinamide adenine dinucleotide + hydrogen, ETC=Electron Transport Chain, NQO1= NAD(P)H quinone dehydrogenase oxidoreductase 1

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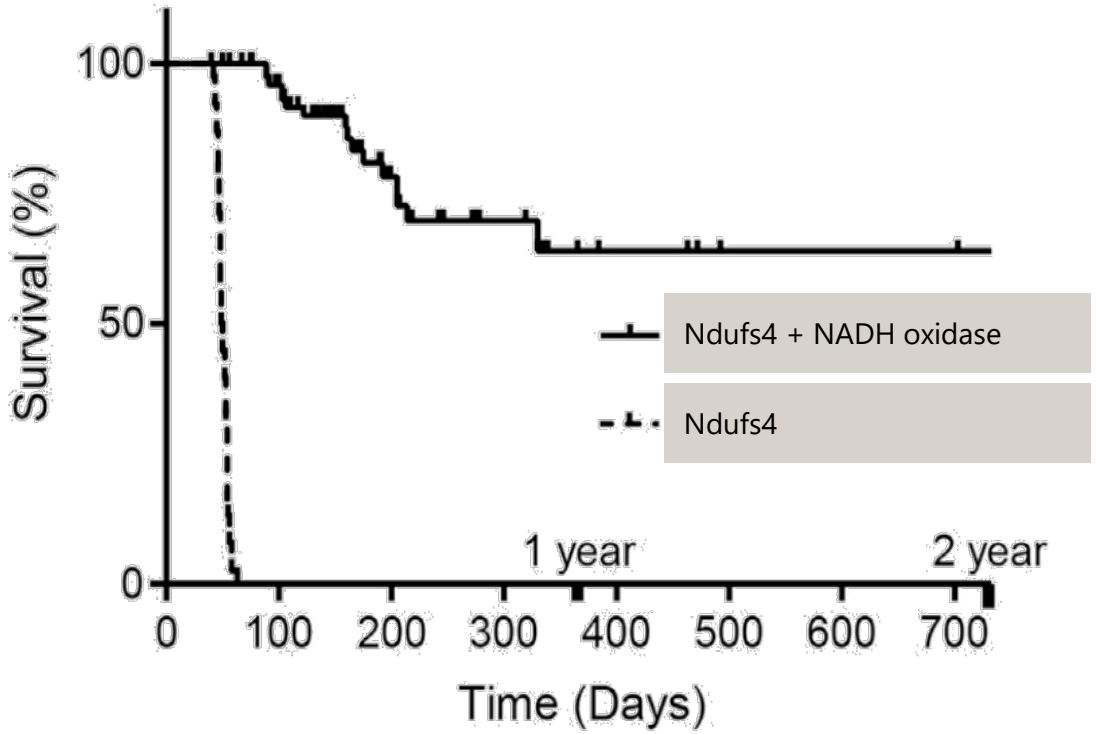
Experimental model validation of NAD⁺/NADH correction in mito disease



Artificial NADH oxidases raises NAD⁺ levels and increases lifespan in mitochondrial disease model (Ndufs4)[1] Other studies show cell metabolism rescue[2] and normalization of mito disease biomarkers[3]

[1] McElroy et al. Cell Metab. 2020;32(2):301-308
[2] Titov et al. Science 2016;352(6282):231-5
[3] Goodman et al. Nature 2020;583:122–126

KL1333 Clinical Program Overview

Incorporating stakeholder input into clinical design

Patients

Fatigue is the most common complaint of mitochondrial disease patients

Myopathy (or muscle weakness) is the second most frequent complaint of patients

Regulators

Recognize the importance of patient input into clinical designs

Support the use of patient reported outcome (PRO) measurement, which have been used increasingly over the last 20 years

PROM as primary endpoints are supported by several precedents across a spectrum of diseases

Payers

All KOLs and Payers stated that the use of a PRO as a primary endpoint is considered acceptable and appropriate

Payers stated that the choice of the primary endpoint would likely not pose reimbursement challenges for KL1333

Payers suggested the use of a second, functional endpoint

Research led to the incorporation of two alternative primary endpoints evaluating the most critical aspects of the disease

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KL1333: Clinical development plan overview

Completed Phase 1a/b

Randomized, Double-blind, Parallel-group, Placebo-controlled, Multiple-site Study

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

A. Single ascending dose study 25mg, with food QD, healthy volunteers.

B. Multiple ascending dose, 5 cohorts (25, 50, 75, 150 and 250 mg) QD, healthy volunteers

C. 10 days dosing, 50 mg QD, 1 cohort, PMD patients

D. Split dosing (75 mg BID or 50 mg TID), 2 cohorts, healthy volunteers

Fatigue endpoint validation study in PMD patients

Regulatory Advice Meetings (FDA, MHRA, EMA)

Ongoing Phase 2 FALCON study

Randomized, double-blind, parallel-group, placebo-controlled

Wave 1 fully recruited

40 patients across

6 countries (US, UK, France, Spain, Belgium, Denmark)

18 sites activated

Wave 2 expansion

180 patients dosed for 48 weeks

Interim analysis (IA)

Wave 1 patients dosed 6 months

Positive outcome achieved

Wave 2 to commence in 2H24

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Efficacy endpoints in Phase 1b are relevant to patients and supported by the FDA

Fatigue Measures

Quality of Life in Neurological Disorders (Neuro-QoL) Short Form (SF) Fatigue

Daily Fatigue Impact Scale (D-FIS)

ePRO Endpoint

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

Neuro-QoL Short Form Fatigue

Score: T-scores are calculated based on points; Maximum 40 points possible

Minimally important differences for fatigue scales ranges from 2.5-5 in T-score*

Myopathy Measure

30 second Sit-to-Stand (30s STS)

Quantitative Endpoint

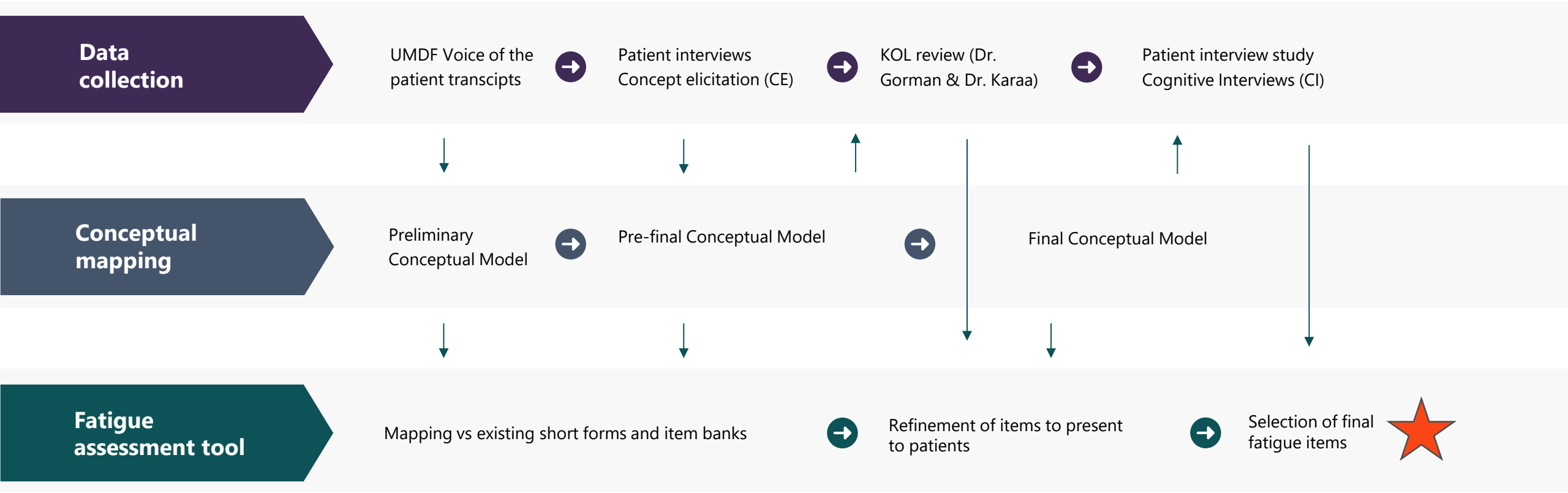
Score = number of repetitions (higher score = better muscle strength/endurance)

≥2 repetitions has been defined as the minimum clinically important difference in osteoarthritis and COPD**

*Yost et al 2011, Nordin et al., 2016

Fatigue patient study created the first mitochondrial disease-specific primary endpoint

Reached regulatory alignment on fatigue endpoint based on validation study in PMD patients



PROMIS® Fatigue Mitochondrial Disease Short Form (see appendix for details)

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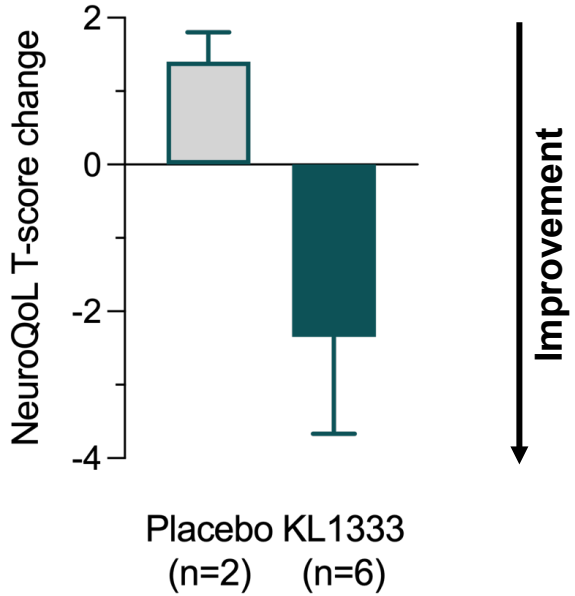
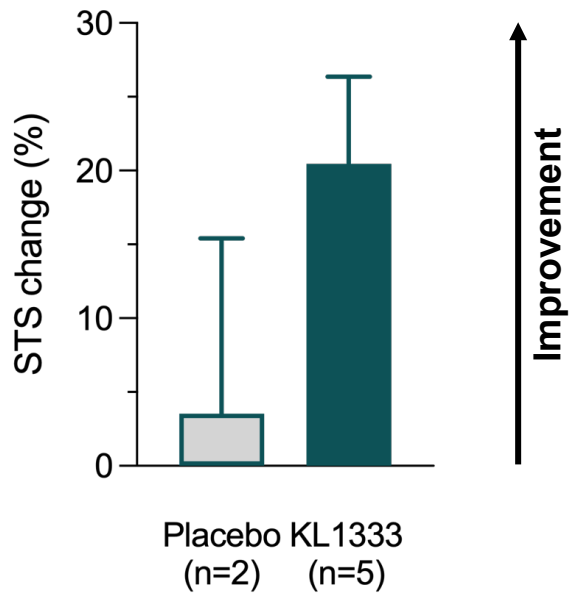
Phase 1b: KL1333 showed signals of efficacy after 10 days, 50 mg/day in mitochondrial disease patients

Reduction of fatigue
Changes from baseline to day 10



Improvement in muscle function
Changes from baseline to day 10



Clinically meaningful effect on fatigue and muscle weakness

Fatigue: Minimally important differences for fatigue scales ranges from 2.5-5 in T-score (Yost et al 2011); DFIS ranges from 3-4 as extrapolated from Nordin et al. 2016. Myopathy: One subject in the KL1333 group did not perform test (excluded from analysis); Minimally important differences for 30sSTS from Wright et al 2011 (Osteoarthritis), Zanini et al. 2019 (COPD)

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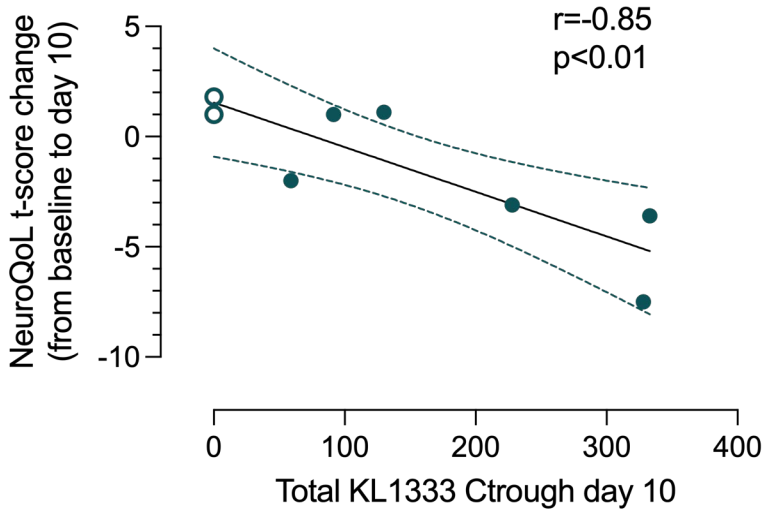
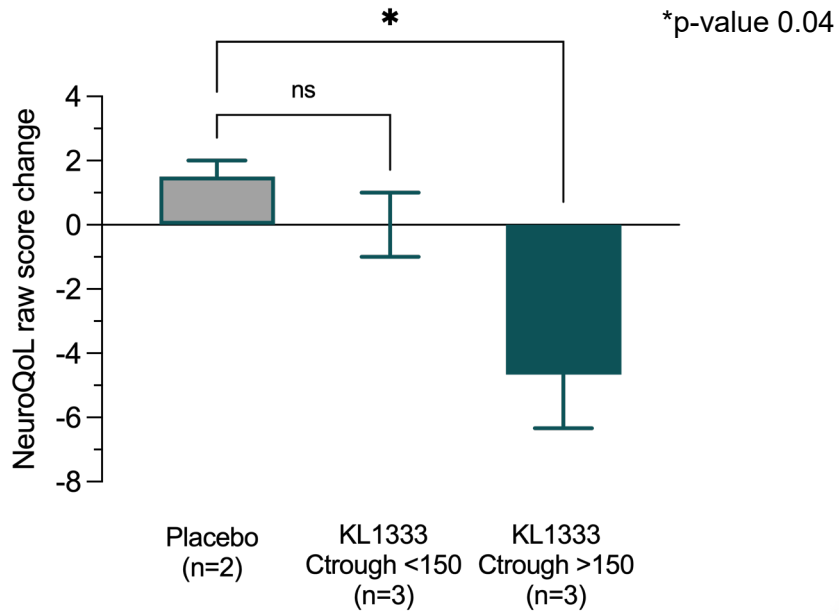
Phase 1b: Correlation between exposure and efficacy exists for all three endpoints

Reduction of fatigue in relation to exposure of KL1333

Neuro-QoL SF Fatigue

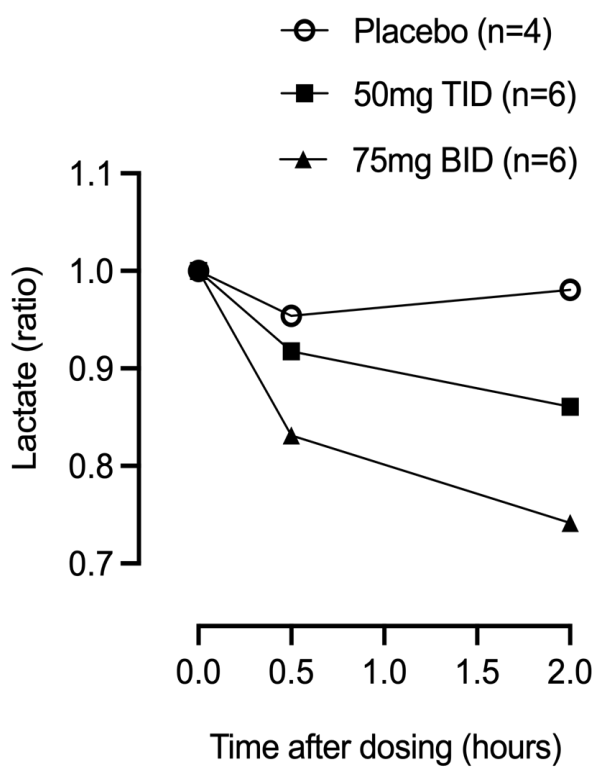


Neuro-QoL SF Fatigue

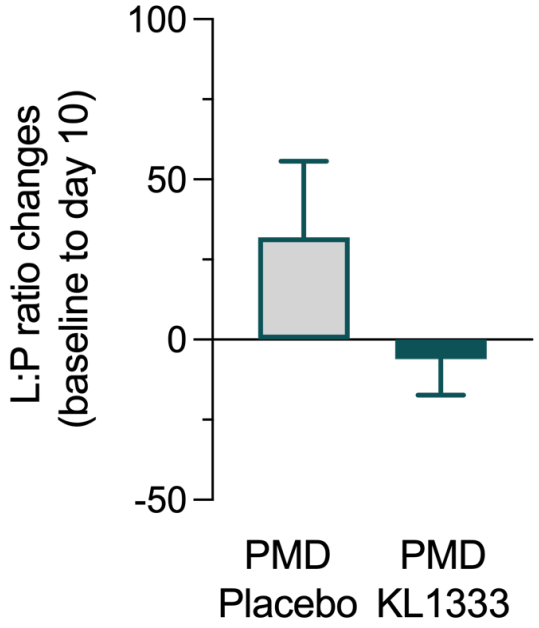


Phase 1b: Target engagement was confirmed with lactate/pyruvate biomarker

Lactate in fasted healthy volunteers



Lactate/ pyruvate ratio in patients



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What is the impact of the Phase 1b data on the KL1333 program?

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 study

- Provides the **first signal of efficacy of KL1333** in mito disease patients
  - **Clinically-relevant endpoints** that are used in the global, potentially pivotal Phase 2 study
  - **Further strengthens the use of fatigue as an endpoint**
- Supports the **inclusion of 30 sec Sit to Stand** (measure of muscle weakness and endurance)
- **Confirmed target engagement using lactate/pyruvate**
- This study, in addition to the drug-drug interaction study, confirm a **good safety profile** for KL1333 with no SAEs in completed studies; gastrointestinal tolerability at high doses

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KL1333 has a very attractive safety profile with no safety signals seen in previous studies

Single ascending dose study 25-800mg

No safety signals

Mild dose-dependent GI AEs at high doses

Drug-Drug Interaction study

KL1333 was a weak inhibitor of CYP1A2 but had negligible effects on other CYP substrates

Multiple dose study 25-250mg + Patient cohort

No safety signals

Tolerability at higher doses limited by mild-moderate GI-related side effects - improved by dividing dose

Similar PK/safety profile in patients compared to healthy volunteers

Signals of target engagement and improvements of fatigue & myopathy

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Clinical development strategy optimized for success

Clinical development plan builds on:

- Deep understanding of the pathophysiology of mitochondrial disease
- Positive efficacy data in placebo-controlled Phase 1b study and no safety signals or SAEs across multiple studies (>100 subjects treated)
- Patient, physician and payor feedback
  - Fatigue is the most common complaint of PMD patients; Fatigue patient study created the first PMD-specific primary endpoint
  - Myopathy (or muscle weakness) is the second most frequent complaint of patients and the second primary endpoint

De-risked potentially pivotal FALCON study is expected to provide a clear path to market in a high value indication

1-year adaptive study design with 180 adult patients

Positive interim analysis at 6 months evaluated conditional power and safety

Two alternate primary endpoints provide two 'shots on goal'

- PMD PROMIS Short form fatigue
- 30 second Sit-to-Stand test

3-month screening period ensures the 'target' patient enters the study

Screening period to confirm the right patients were included in the study, including genetically – and phenotypically-defined subgroups of PMD

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Wave 2 expansion

180 patients dosed for 48 weeks

Interim analysis (IA)

Wave 1 patients dosed 6 months

Positive outcome achieved

Wave 2 to commence in 2H24

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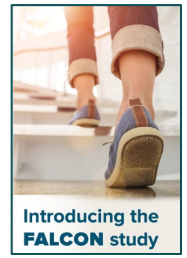
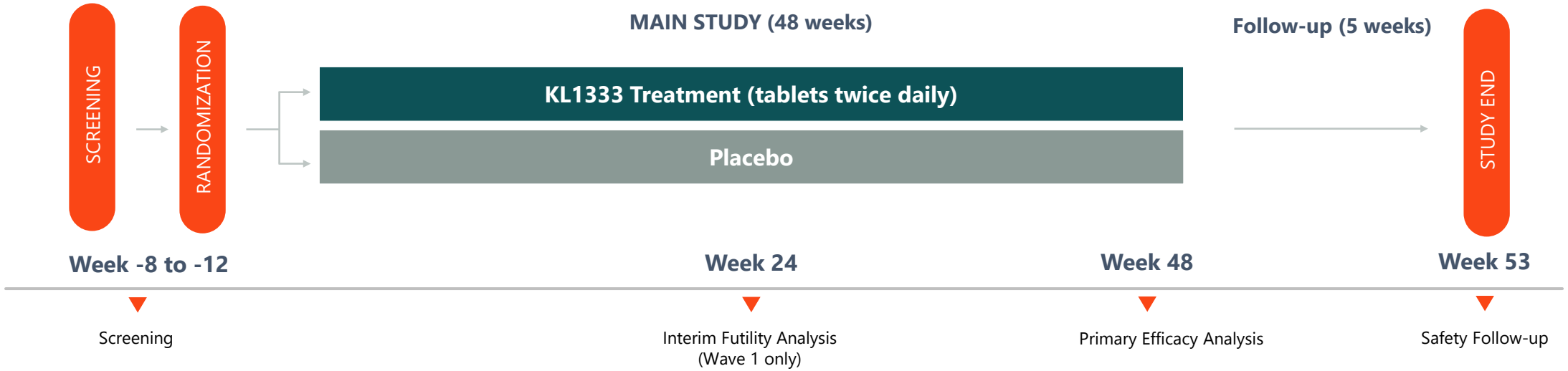
Pivotal FALCON study commencing final wave of study



Study is powered for potential success with positive interim analysis outcome



PARTICIPANTS

Adult Primary Mitochondrial Disease (PMD) patients with:

Multisystemic mitochondrial DNA-related disease

Disease expressions involving at least chronic fatigue and mitochondrial myopathy/exercise intolerance

INTERVENTION

Oral twice daily dosing (50mg-100mg) for 48 weeks (including dose titration phase)

PIVOTAL STUDY DESIGN

Randomized, double-blind, parallel-group, placebo-controlled (40% placebo, 60% active)

Sample size: 180 patients (interim analysis following recruitment of 40 patients in Wave 1)

Alternate Primary Endpoints: Fatigue (validated for PMD*), 30 Second Sit-to-Stand

Secondary: Neuro-QoL Lower Extremity Function; 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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Abliva is extremely pleased with the IA outcome.

- Both primary endpoints passed futility. The result confirms that both independent, alternative endpoints have the potential to be successful, but only one of which is required to file for approval.
- By performing the sample size re-estimation and increasing the sample size of the study based on the weakest endpoint, the power of both endpoints has been increased, making a positive study more likely.
- The strong safety profile of KL1333 has been confirmed following long-term dosing over 24 weeks, a major step forward from the previous phase 1b study with 10 days of dosing.
- The dosing strategy with evaluation of the maximally well tolerated dose in each patient has performed as expected, allowing us to test different doses without increasing the size of the study (and helping to keep the patients in the study).
- Study patients demographic profile confirms the assumptions from previous natural history studies and positions the project well for future label claims.
- Abliva is the first company in the mitochondrial disease space to readout a positive interim analysis, and thus this is an important de-risking event for investors and partners considering our strong development program.

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Team is focused on ensuring KL1333 is ready for NDA submission at the end of FALCON

- Favorable Safety Profile
- Early Signs of Efficacy
- Facilitated Regulatory Path
- Strong IP Protection
- Manufacturing for Launch Initiated
- Ongoing Pivotal Recruiting to Plan
- Non-Clinical Package Discussions Planned
- Launch Plan Kickoff after Interim Analysis

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NV354 Program Overview

Opportunity to address mito disease-associated neurological complications in pediatric patients

- Children diagnosed with mitochondrial disease are impacted by neurological symptoms (neurodevelopmental delays, neuromuscular weakness, ataxia, cognitive development, ophthalmologic problems) and often have a greatly reduced life span
- Second program in the portfolio is a brain-penetrable energy replacement therapy that modifies disease progression
- Pediatric target populations include Leigh syndrome, MELAS or Leber hereditary optic neuropathy (LHON)
- Other potential indications include Parkinson's disease and indications which could benefit from mitochondrial energy booster

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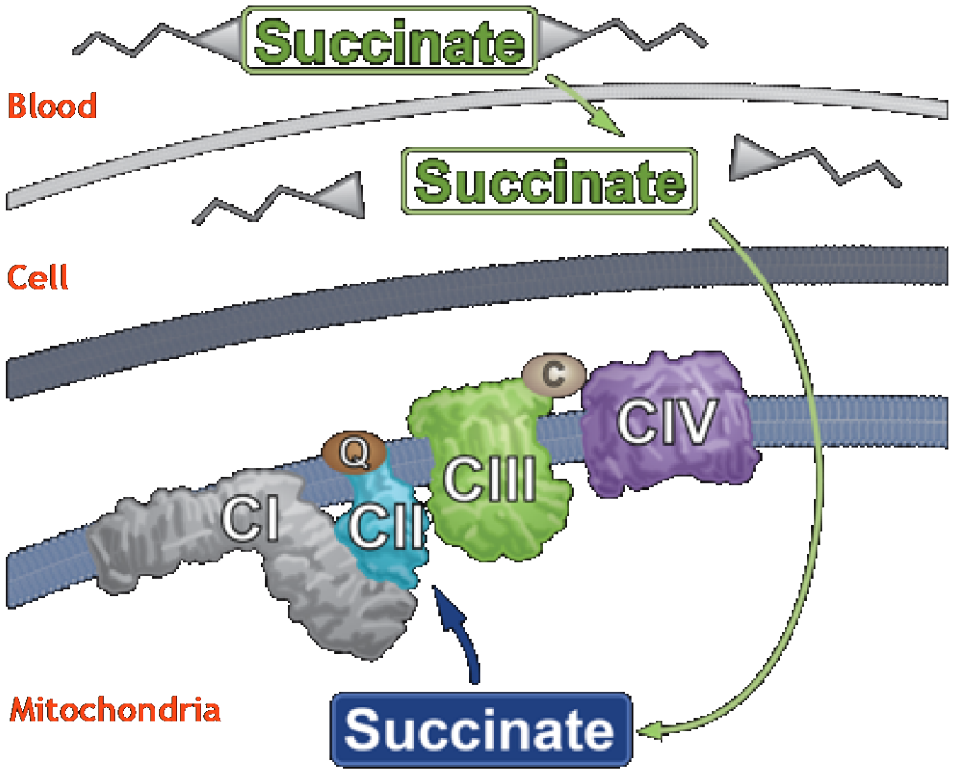
NV354 is a brain-penetrable prodrug of succinate

- Designed by Abliva scientists
  - Strong patent protection (3 families)
  - Orphan drug designation (US, EU)
- Disease-modifying energy replacement therapy
- Targets neurological diseases with a defective Complex I in the electron transport chain (ETC)
- Phase 1 ready following UK regulatory (MHRA) scientific advice

Succinate pro-drug bypasses defective Complex I



- Complex I dysfunction is one of the most common causes of mitochondrial disease
  - Key factor in Leigh Syndrome, MELAS, and LHON
- Disease modifying potential
  - Protects mitochondria and loss of organ function
  - Prevents complications caused by acute energy crisis
- Succinate enters Complex II of the electron transport chain, allowing production of ATP when Complex I is dysfunctional
- Succinate pro-drug is protected by a strong patent estate and Orphan Drug Designation (US, EU)

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

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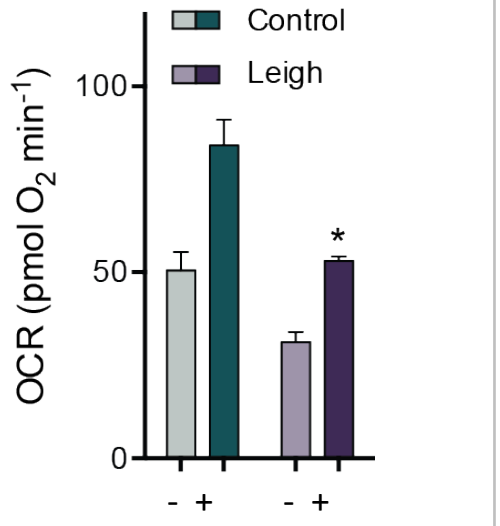
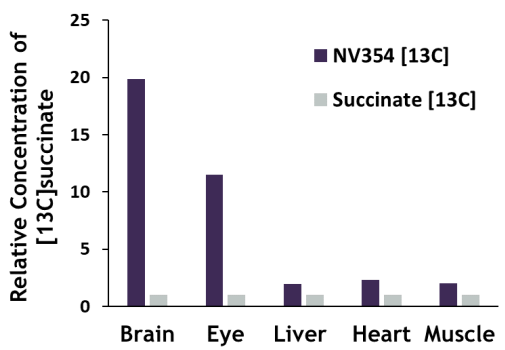
NV354 corrects underlying biochemical dysfunction in Complex 1 deficient patient cells



Improved mitochondrial function in Leigh patient cells with succinate prodrug



^{13}C-labelled succinate delivery to organs

- NV354 has optimized drug properties
  - High oral bioavailability
  - High brain distribution
  - Favorable tolerability in toxicology studies
- Bypasses Complex 1 (most common mutation in Leigh Syndrome)
- Normalizes function of mitochondria in cells from Leigh patients

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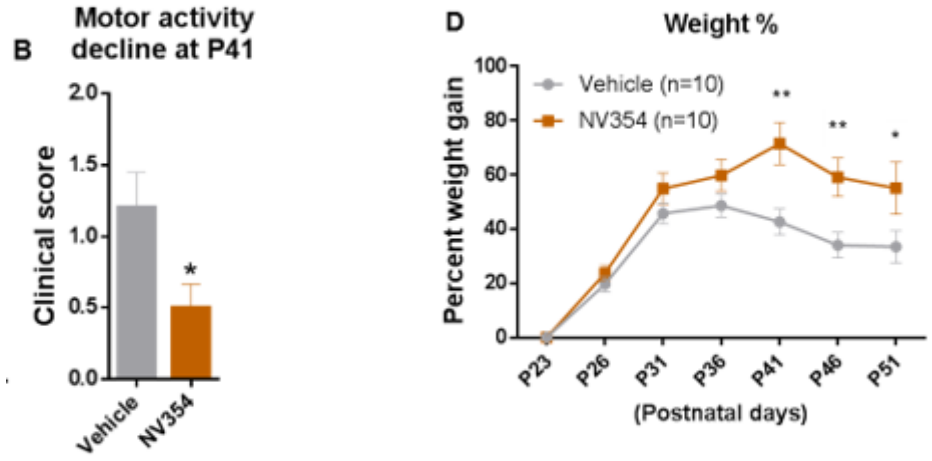
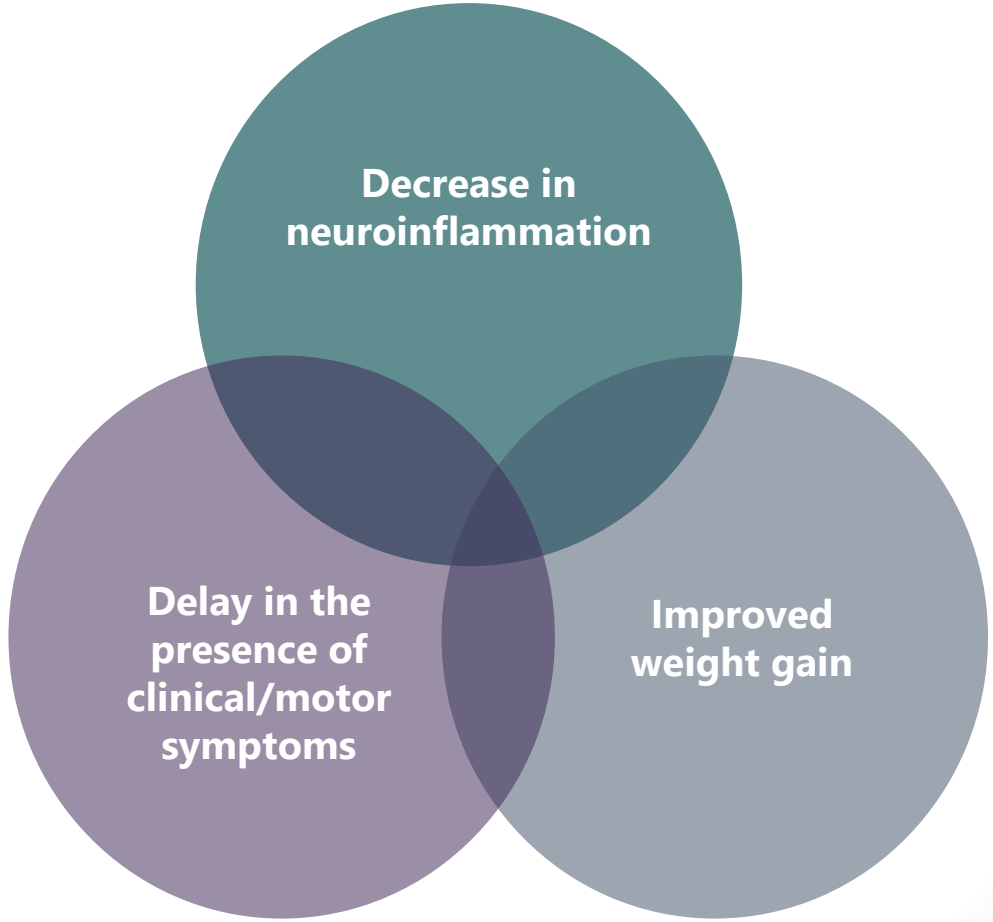
Disease model study suggests impact on brain inflammation, motor activity and weight

Ndufs4 model with Complex I dysfunction





Unpublished data; Quintana lab, UAB (Barcelona)

First-in-class NV354 is ready for Phase 1

In summary, NV354:

- Aims to address neurologic issues in pediatric mitochondrial disease patients with diseases such as MELAS, Leigh, LHON
- Is a succinate prodrug that corrects underlying biochemical dysfunction in Complex I of the electron transport chain
- Orally bioavailable, penetrates the brain and shows good tolerability in preclinical animal models.
- Demonstrated efficient delivery of succinate to organs after i.p. or oral administration in mice.
- Efficacious in toxin-induced Complex I dysfunction in rat and genetic mitochondrial disease model in mice.
- Phase 1-ready as confirmed by MHRA scientific advice meeting; Novel development options being considered to shorten time and cost to patient data

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Summary

The Abliva portfolio summary: two rare disease, first-in-class programs

Lead program, KL1333, commenced pivotal Phase 2 study in late 2022

- 12-month adaptive design study; 180 adults with mtDNA mutations with systemic disease, fatigue and muscle weakness
- Positive Interim analysis in July 2024 de-risked pivotal study design with two alternative endpoints and confirmed strong safety profile
- Positive efficacy data in placebo-controlled Phase 1b study
- No safety signals or drug-related SAEs across multiple studies (>130 subjects treated)
- High unmet need with >$1bn blockbuster peak sales potential
- First-in-class mechanism with strong patent protection, ODD in US, EU, Fast-track status in US

Pipeline asset NV354, ready for Phase 1, targets neurologic diseases

Roadmap

2023 - 2024 Key Accomplishments

2023

- NV354 patent granted in the U.S. in February
- NV354 received Orphan Drug Designation in the U.S. in April
- First patient dosed in KL1333 FALCON study achieved in June
- KL1333 received Fast Track Designation in September
- NeuroSTAT® for Traumatic Brain Injury outlicensed to Owl Therapeutics in November
- NV354 received Orphan Drug Designation in the E.U. in December
- All patients recruited into Wave 1 of the FALCON study in December

2024

- Financing completed to elongate runway around IA
- FALCON Wave 1 patients complete 24 weeks of dosing
- FALCON interim analysis completed

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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

30+ years of research in mitochondrial medicine

Dag Nesse

VP Clin Ops

Abliva since 2023

Previous Head of Clin Ops at Calliditas

25 years of Clinical Ops incl. leadership from pivotal study to data readout, marketing authorization, and launch

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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

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Investment Highlights

Novel therapy targets underlying pathology of mitochondrial disease

- Novel therapeutic approach **modulates underlying pathophysiology** to promote mitochondrial biogenesis and restore energy metabolism
- Supports symptom reduction and disease modification in primary mitochondrial disease

Attractive commercial opportunity addresses high unmet need

- No approved medicines for systemic PMD
- **Opportunity for >1B+ US, EU peak sales**
- Lead program KL1333 is poised to be the first to market treatment for mitochondrial disease

Pivotal FALCON trial of KL-1333 ongoing with well-defined path forward

- **Positive interim analysis confirms safety and two 'shots on goal' strategy**
- Phase 2 optimized for success, incorporates patient input and builds on strong efficacy and favorable safety data
- KL1333 poised for NDA submission following FALCON completion

First-in-class assets with strong regulatory and IP packages

- **KL1333: Fast Track Designation in US, Orphan Drug Designation in US and EU**
- NV354: Phase 1 ready in neurological diseases; Orphan Drug Designation in the US, EU
- Additional discovery programs in development with opportunities to drive long-term value

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Appendix

PROMIS® Fatigue Mitochondrial Disease Short Form

	In the past 7 days…	Never	Rarely	Sometimes	Often	Always
FATEXP5	How often did you experience extreme exhaustion?	☐	☐	☐	☐	☐
FATEXP18	How often did you run out of energy?	☐	☐	☐	☐	☐
FATEXP20	How often did you feel tired?	☐	☐	☐	☐	☐
FATEXP26	How often were you too tired to enjoy life?	☐	☐	☐	☐	☐
FATIMP3	How often did you have to push yourself to get things done because of your fatigue?	☐	☐	☐	☐	☐
FATIMP19	How often were you too tired to do your household chores?	☐	☐	☐	☐	☐
FATIMP21	How often were you too tired to take a bath or shower?	☐	☐	☐	☐	☐
FATIMP29	How often were you too tired to leave the house?	☐	☐	☐	☐	☐
FATIMP30	How often were you too tired to think clearly?	☐	☐	☐	☐	☐

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