

Efficacy of KL1333 in Adult Patients With Primary Mitochondrial Disease

NCT05650229

Status	RECRUITING
Phase	Phase 2
Sponsor	Abliva AB
Enrollment	180 participants

Key Eligibility Criteria

Inclusion (24)

- Age 18 years or older.
- A confirmed PMD diagnosis caused by a known pathogenic gene mutation or deletion of the mitochondrial genome (category 6 of the International Classification of Inborn Metabolic Disorders \[ICIMD\]¹² according to American College of Medical Genetics (ACMG)/Association of Molecular Pathology (AMP) criteria¹, with multisystemic disease expressions, including:
- 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).

... and 19 more (see full listing online)

Exclusion (23)

- Primary mitochondrial disease with predominant neurodegenerative phenotypes, such as, but not limited to, Leigh syndrome, Leber hereditary optic neuropathy (LHON) and Neuropathy ataxia-retinitis pigmentosa syndrome (NARP).
- Primary mitochondrial disease nuclear DNA mutations or mutations causing mtDNA destabilisation. Genetic mtDNA variants of uncertain significance, likely pathogenic, or pathogenic mutations with degrees of heteroplasmy below what can be considered to definitely cause PMD.
- General fatigue or muscle weakness due to causes other than mitochondrial disease, in the opinion of the investigator.
- Significant cardiovascular disease (e.g., sustained or symptomatic arrhythmia; dilated heart chambers or reduced function; Mobitz II atrioventricular block or greater) OR abnormal ECG that is clinically significant, as determined by the investigator. Any QTcF \geq 450 msec for male patients and \geq 470 msec for female patients is exclusionary. In the case of an exclusionary QTcF, the ECG can be repeated twice and the average of 3 QTcF intervals should be used to determine the QTcF eligibility.
- Recent history of unstable disease, inadequately controlled neurological manifestations or not recovered from stroke-like episodes including but not limited to:

... and 18 more (see full listing online)

Locations (23 total)

Akron Children's Hospital, Akron, Ohio, United States
The University of Texas Health Science Center at Houston, Houston, Texas, United States
Hopital Universitaire de Bruxelles (H.U.B)/ Academisch Ziekenhuis Brussel, Brussels, Belgium
... and 20 more locations

<https://clinicaltrials.gov/study/NCT05650229>

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