Abliva

First Positive Human Efficacy Signals For KL1333

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Today, Abliva has reported Q121 results alongside positive human efficacy signals in their phase 1a/b trial for KL1333 in primary mitochondrial disease (PMD). This is the first human efficacy data that Abliva have reported for their lead asset and as such is an encouraging and important development. The positive data was generated by a cohort of eight PMD patients within the study, six of whom were dosed with KL1333 and two with placebo. This cohort was not powered to show superiority, but the patients that received KL1333 had numerically superior improvements in two patient-reported fatigue endpoints and the 30-second Sit to Stand endpoint. Importantly, a dose-dependent effect was found, implying that KL1333 was the likely driver of those differences. Only 10 days of dosing was carried out, so this data provides evidence that KL1333 also acts rapidly. No serious adverse safety signals were found in the trial (in both healthy volunteers or the eight person PMD cohort) and further supportive pharmacokinetic data was generated. Management will now review the totality of this data before finalising the clinical protocol of the pivotal phase 2/3 study, which is expected to initiate in the second half of this year. Click [HERE] to download our initiation from January 2021.

Q121 Results

There have been several significant developments during the first three months of the year, including the appointment of Ellen Donnelly as new CEO, the readout of the KL1333 P1 clinical study as explained above, the raising of SEK 80m in funding from a share issuance and the ending of the license agreement with Fortify (to which we ascribed zero value in our initiation).

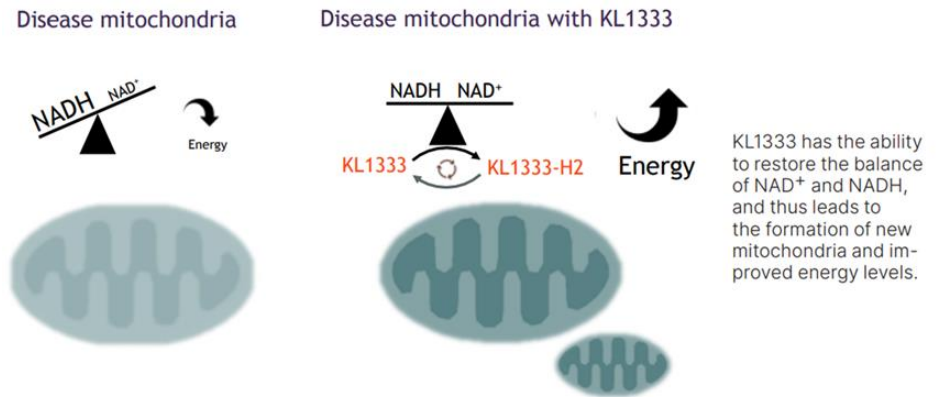
KL1333

KL1333 is an orally administered small organic molecule derived from β-lapachone, a quinone-containing compound obtained from the Lapacho tree. It is in development for the treatment of adults with primary mitochondrial disease within the spectrum of MELAS-MIDD and CPEO-KSS, which together account for ~40% of PMD. These diseases cause a wide range of severe symptoms, most particularly fatigue and there are around 40,000 patients in the US and Europe. The drug holds Orphan Drug Designation in both regions. Abliva in-licensed KL1333 from Yungjin Pharm and have exclusive global rights for the drug, excluding Korea and Japan.

KL1333 treats mitochondrial disease by increasing the concentration of NAD+ to normal levels. This increased NAD+/NADH ratio has a two-pronged effect which helps to treat the underlying condition by increasing ATP production and increasing mitochondrial biogenesis.

Based on our assumptions, we believe the drug could generate up to $150m/year ($750m/year when fully de-risked) and we value it at SEKM1.90/share based on a 2024 launch.

Chart 1: KL1333 Mechanism of action



Source: Company reports

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Full 12-month historical recommendation changes are available on request

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