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# Khondrion presents Phase II KHENERGY trial data

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Clinical stage pharma company, Khondrion focusing on small molecule therapeutics for mitochondrial diseases has announced results from its KHENERGY study.

This study is Phase II exploratory trial with oral KH176 in the m.3243A>G multisystem mitochondrial MELAS and MIDD syndromes and mixed phenotypes. The results of the trial were presented by Prof. Jan Smeitink, Khondrion's CEO, at the Dutch Life Sciences Conference.

"The final reporting of the KHENERGY study is planned for Q1 2018, but encouraged by the results, we wanted to share these preliminary data regarding safety and efficacy now", said Jan Smeitink.

Patients received KH176 in a 100 mg twice-daily oral dosing schedule for one month. Efficacy endpoints included objective, quantitative assessments as well as questionnaires evaluating the mood and quality of life of patients. The study also explored biomarkers associated with mitochondrial functioning.

Dr. Edwin Spaans, Khondrion's Chief Medical Officer said, "The preliminary findings of this study related to adverse events showed a promising safety profile. Also, the pharmacokinetic analysis of KH176 showed that the candidate drug's maximum blood concentrations remained below the pre-defined safety threshold obtained in Phase I evaluations."

Of the functional outcomes measures, two aspects of alertness showed positive trends. All others, did not show a positive signal in the 4 weeks treatment arm.

With regard to clinical outcomes, statistically significant improvements were observed in the total Beck Depression Inventory score and its affective sub-domain. Positive trends were observed in the HADS depression subsection and the RAND-36 SF affective symptoms. Self-reported outcomes revealed an amelioration of migraine in three out of three affected subjects.

Dr. Mirian Janssen, Principal Investigator and Chris Verhaak, Clinical Psychologist of the KHENERGY study said, "Given the relatively short duration of this study, these findings are encouraging."

“Based on the outcome of the Phase II study, we have decided to immediately continue with all necessary steps enabling the next phases of our KH176 development program, including all Phase III preparations”, said Jan Smeitink.

Based on the outcome of the KHENERGY study the company continues preparing for a pivotal program to confirm the potential benefits of KH176 in patients with mitochondrial disease.