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## **Khondrion presents Phase II KHENERGY trial data**

### ***Data supporting Phase III development of KH176 in mitochondrial disease***

NIJMEGEN, The Netherlands – Khondrion, a leading clinical-stage pharmaceutical company focusing on small molecule therapeutics for mitochondrial diseases, today announced results from its KHENERGY study, a 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.

The KHENERGY study is a Phase II, single-center, double blind, randomized, placebo-controlled 2-way crossover trial involving 20 patients. 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.

“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,” said Dr. Edwin Spaans, Khondrion’s Chief Medical Officer.

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.

“Given the relatively short duration of this study, these findings are encouraging”, according to dr. Mirian Janssen, Principal Investigator and Chris Verhaak, Clinical Psychologist of the KHENERGY study.

“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.

## About KHENERGY and KH176

The KHENERGY study is a Phase II, single-center, double-blinded, randomized, placebo-controlled 2-way cross over trial involving 20 patients harboring the m.3243A>G mutation in the mitochondrial genome. The study is supervised by dr. Mirian Janssen (MD, PhD), of the Radboud Center for Mitochondrial Medicine at the Radboudumc, Nijmegen, The Netherlands.

KH176 is an orally bio-available small molecule in development by Khondrion for the treatment of mitochondrial (-related) diseases. The compound is a member of a new class of potential Khondrion drugs essential for the control of oxidative and redox alterations. Khondrion reported earlier that KH176 demonstrated a favorable pharmacokinetic profile and an acceptable safety profile in randomized, placebo-controlled, double blind Phase I clinical trials, performed in healthy male volunteers. The results of this study were recently published in the Orphanet Journal of Rare Diseases.

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.

## About Khondrion

Khondrion is a privately held leading clinical-stage pharmaceutical company focusing on developing small molecule therapeutics for mitochondrial (-related) diseases. The potential of several lead compounds to serve as new treatment modalities for mitochondrial disease is currently being explored. Khondrion's KH176 has been granted Orphan Drug Designation (ODD) for Leigh disease and MELAS syndrome in Europe and for all inherited mitochondrial respiratory chain disorders in the USA. Khondrion has established collaborations with patient organizations, patient advocacy groups, university expert centers and research groups around the world as well as with small, medium and large enterprises. The company is supported by the Dutch Foundations Energy4All, Join4Energy, Road4Energy, Ride4Kids, Tim Foundation, Zeldzame Ziekten Fonds, and National and European Governments. Khondrion has a strong intellectual property position protecting its emerging product portfolio via granted and multiple, broad patent applications. For more information, please visit [www.khondrion.com](http://www.khondrion.com)

## Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements. All statements, other than statements of historical facts, contained in this press release, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions

are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements represent our views only as of today and should not be relied upon as representing our views as of any subsequent date. If underlying assumptions prove inaccurate or known or unknown risks or uncertainties materialize, actual results could vary materially from the expectations and projections of Khondrion. Risks and uncertainties include, but are not limited to: challenges and uncertainties inherent in product development, including the uncertainties of clinical success and the timeline for the availability of KH176. While we may elect to update these forward-looking statements at some point in the future, we specifically disclaim any obligation to do so, even if our views change.