bringing energy to life

about Khondrion

Khondrion is a clinical-stage pharmaceutical company discovering and developing therapies targeting mitochondrial disease. Its team of scientists are advancing the company's proprietary science through a wholly-owned clinical and preclinical small molecule pipeline of potential medicines.

Cellular consequences like abnormal mitochondrial architecture, reactive oxygen species production and alterations in the cellular redox-state are common findings in mitochondrial diseases. Khondrion's drug development strategy is based on counteracting these cellular consequences to stop disease progression and to restore normal cellular function.

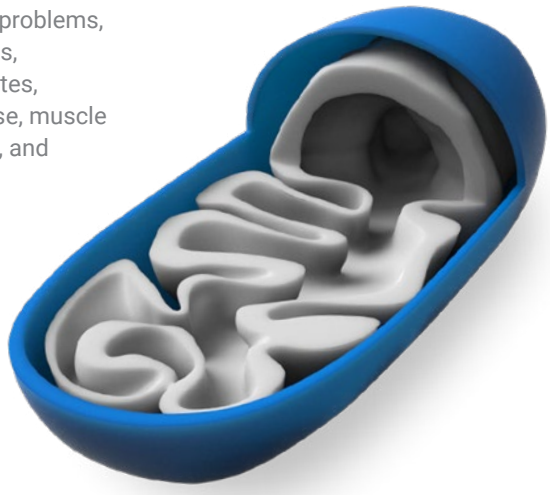
Founded: 2012
Headquarters: Nijmegen, The Netherlands

mitochondrial disease

Mitochondrial disease occurs when mitochondria, found within all cells of the human body and responsible for producing the energy necessary for life, are defective. This can result in a wide range of serious and debilitating illnesses, signs and symptoms of which can include: cognitive problems, learning disabilities, blindness, deafness, heart failure, diabetes, fatigue, intolerance to exercise, muscle weakness and gait problems, and stunted growth.

Examples of mitochondrial disease include:

- Leigh disease
- MELAS (mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes) spectrum disorders
- MIDD (maternally inherited diabetes and deafness)
- LHON (Leber's hereditary optic neuropathy)
- Other respiratory chain/ oxidative phosphorylation disorders



KHONDRION

leadership team

Jan Smeitink, MD, PhD
Chief Executive Officer & Founder

- Recognised as a world leader in Mitochondrial disease with more than 25 years experience in patient care, diagnostics and research
- Professor in mitochondrial medicine, has published more than 390 peer-reviewed scientific papers

Julien Beyrath, PhD
Chief Scientific Officer

- Over 10 years experience in drug discovery and pre-clinical development, focused on the identification of new drug targets for mitochondrial disease
- Strong academic and industrial life-sciences network
- Responsible for Khondrion's internal and external research and development programmes

Dennis Lammers, MBA
Chief Financial Officer

- Financial specialist with long-standing experience in the management and leadership of life-science companies
- Holds a position in the executive management of the Radboud University Medical Center Holding, the Netherlands

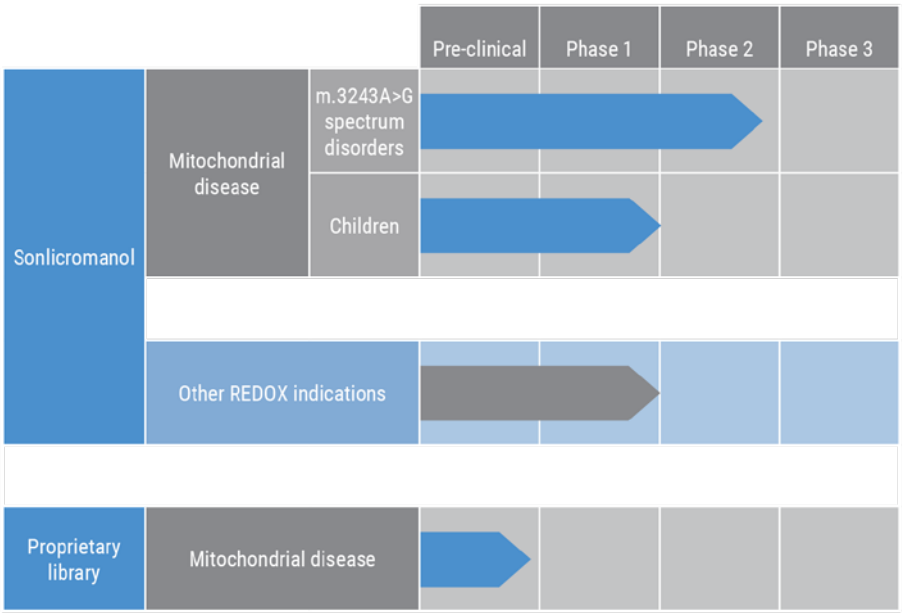
Rob van Maanen, MD
Chief Medical Officer

- Over 20 years' experience in strategic and operational global drug development, medical affairs, pharmacovigilance and regulatory affairs
- Responsible for clinical development of lead asset, Sonlicromanol, and oversees Khondrion's broader clinical strategy and medical operations

Arnout Ploos van Amstel
Chief Operating Officer

- Executive leader in the life sciences and biotechnology industries with more than 30 years of business and operations experience
- Previously SVP, Head and General Manager of Novartis' Immunology, Hepatology and Dermatology Global Business Franchise

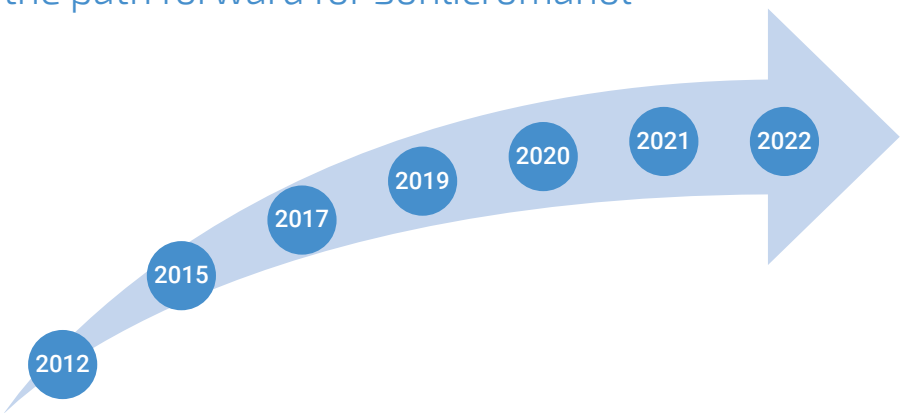
advancing a proprietary pipeline



Khondrion's lead asset – Sonlicromanol

- Potential first-in-class oral small molecule
- Novel dual mode of action
- Well tolerated in phase IIa studies with early indications of potential impact on clinical symptoms
- Currently in phase IIb clinical development to treat a range of mitochondrial diseases including MELAS
- Orphan Drug Designation for MELAS and Leigh disease in Europe and for all inherited mitochondrial respiratory chain disorders in the USA
- Strong IP protection
- Potential for indication expansion in both orphan and non-orphan diseases

the path forward for Sonlicromanol



Develop-ment begins	Phase I completed	Phase IIa completed	KHENER-GYZE study begins	Pivotal study	Paediatric expansion	Further indication expansion

For more information visit www.khondrion.com | Contact details info@khondrion.com

further pipeline innovation

Preclinical research is underway exploring the potential of Khondrion's pipeline to target diseases resulting from a cellular reduction–oxidation (redox) imbalance.

Redox imbalance occurs when the redox signaling processes in cells – the fundamental processes by which cells detect damage and then start their repair mechanisms – are not controlled. Diseases currently under investigation include genetic forms of Parkinson's disease, congenital muscular dystrophy and inborn errors of metabolism.

KHENERGYZE

Ongoing phase IIb study, partly funded by the H2020 SME instrument phase 2, to investigate the true treatment effect on mitochondrial disease related symptoms, evaluating the effect of Sonlicromanol on attention domains of cognitive functioning (composite of attention/alertness related tasks)

A double-blind, randomized, placebo-controlled, multi-center, three-way cross-over study with two doses of Sonlicromanol versus placebo in patients with a genetically confirmed m3243A>G mutation
27 adult patients including but not limited to MELAS, MIDD and mixed phenotypes, 9 subjects in each group dosed twice daily (50 or 100 mg) over 28 days

partnering and collaboration

Khondrion has partnerships across a clinical and academic network, and works closely with stakeholders to ensure its research programmes are always focused on patients' needs. If you are interested in being part of this, if you're involved in work that may be relevant to Khondrion or if you would simply like to learn more about our research, then get in touch.

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