

# Press Release

26 June 2019



## **Katairo Announces “First Patient Treated” in STARTT Study of Remofuscin®.**

**STARTT: Stargardt Remofuscin® Treatment Trial: A multi-national, multi-center, double-masked, placebo-controlled, proof of concept trial to evaluate the safety and efficacy of oral soraprazan in adults with Stargardt disease.**

*Tübingen, June 26, 2019*

Katairo GmbH, a biopharmaceutical company focused on discovering, developing and commercializing novel treatments for back of the eye diseases, today announced the treatment of the first patient in the company’s placebo-controlled study of Remofuscin in adults with Stargardt disease.

“Stargardt disease is a blinding disease by legal standards with no approved treatment and among the most common retinal dystrophies caused by a single gene. It is associated with accumulation of lipofuscin in the retinal pigment epithelium”, said Wolfgang Klein, Chief Executive Officer of Katairo. “Accumulation of lipofuscin is associated with a number of negative effects for the RPE and photoreceptors, ultimately it may lead to retinal degeneration. Remofuscin was found to remove lipofuscin from RPE in various non-clinical models, including non-human primates. Stargardt patients may benefit from Remofuscin®.”

“We are very excited to participate in the STARTT study and to have the first patient treated in our trial center at the Radboud University Medical Center” says Prof Carel Hoyng, lead principal investigator in this multinational study funded by the European Commission. “This placebo-controlled study will evaluate Remofuscin® in adults with Stargardt disease. The primary endpoint is to assess removal of lipofuscin as measured by quantitative autofluorescence – a biomarker for lipofuscin. Secondary endpoints include the evaluation of safety and efficacy of the treatment.”

The trial will enroll approximately 90 Stargardt disease patients in up to four European countries including the Netherlands, Germany, Italy and the UK. More information about the trial is available in the clinical trials register at [www.clinicaltrialsregister.eu/ctr-search/trial/2018-001496-20/NL](http://www.clinicaltrialsregister.eu/ctr-search/trial/2018-001496-20/NL) (EudraCT Number 2018-001496-20).

The clinical trial is scheduled to end in 2021.

### **About Stargardt disease**

Stargardt disease (STGD) is an orphan indication and genetic eye disorder that causes progressive vision loss leading to blindness by legal definition. The disorder affects the macula, an area of the retina responsible for color and sharp central vision. Individuals with the condition have abnormal accumulation of lipofuscin in the cells of the retinal pigment epithelium (RPE). Lipofuscin is auto-fluorescent and readily discernible with non-destructive/non-invasive, ophthalmic imaging methods. In advanced stages of STGD, the build-up of lipofuscin in the RPE results in the loss of the RPE. As the RPE provides support to photoreceptors, the overlying photoreceptors are lost too. People with STGD can also have problems with night

vision and color vision. The current treatment options are very limited and focus on preventive measures to avoid excessive light exposure.

### **About Remofuscin**

Soraprazan, the active ingredient of Remofuscin®, was initially in development as an acid pump antagonist (APA) to treat gastro-esophageal reflux disease (GERD). The small molecule completed Phase I and Phase II clinical trials. Interestingly, soraprazan was also observed to result in removal lipofuscin of the RPE in non-human primates treated with Soraprazan.

The evidence in support of Remofuscin® as a potential medicinal product candidate for the treatment of STGD is compelling. Katairo GmbH has licensed data and related IP from the earlier developer. Remofuscin is protected by patents and orphan designation for use of soraprazan for the treatment of STGD.

### **About funding and collaborators**

This project has received funding from the European Union's Horizon 2020 Research and Innovation program under Grant Agreement No 779317 "Soraprazan - a new regenerative therapy for Stargardt's disease".

The recipient of this grant is a consortium formed by the following institutions and principle investigators:

- Eberhard-Karls-Universität Tübingen, (Coordinator and trial site), Dr. Katarina Stingl
- Radboud University Medical Center, Nijmegen (trial site), Prof. Dr. Carel Hoyng
- Ospedale San Raffaele, Milano (trial site), Dr. Maurizio Battaglia Parodi
- Leiden University Medical Center (trial site), Prof. Dr. Camiel Boon
- Southampton University Hospital (trial site), Prof. Dr. Andrew Lotery
- Smerud Medical Research, Oslo (clinical research organization)
- Katairo (sponsor)

### **About Katairo**

Katairo is a biopharmaceutical company focused on the development treatments for back of the eye indications. Katairo's lead development is Remofuscin® for the treatment of Stargardt Disease (phase 2). Katairo will also develop Remofuscin for the treatment of dry age-related macular degeneration (phase 2). For more information, visit [www.katairo.com](http://www.katairo.com).

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