Press Release

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Remofuscin® prevented retinal thinning in Stargardt disease patients. First results from the STARTT Study were presented at EURETINA in Amsterdam.

Tübingen, October 6, 2023

Katairo GmbH, a biopharmaceutical company focused on discovering, developing and commercializing novel treatments for back of the eye diseases, today announced topline results of the STARTT study. The Stargardt Remofuscin Treatment Trial (STARTT) was a 2-year study comparing Remofuscin® tablets to placebo.

The primary endpoint, quantitative autofluorescence (qAF), a novel method to quantify retinal autofluorescence, proved highly variable and did not show significant changes from baseline in both study groups. However, Remofuscin® treated patients had significantly less retinal thinning on tomographic images (SD-OCT) compared to placebo. This protective effect increased over time and was observed for both eyes. The beneficial outcome of Remofuscin® treatment was observed in several retinal structures, most notably in the Outer Nuclear Layer (ONL) and Ellipsoid Zone. Depletion of these is closely associated with progression in Stargardt disease and for both at least one eye progressed significantly less with Remofuscin® treatment after two years ($p < 0.05$). The data from the functional endpoints of Best Corrected Visual Acuity (BCVA) and Mesopic Microperimetry (mMP) after two years support the anatomical findings.

Dr Wolfgang Klein, Managing Director of Katairo said, “We are very pleased with the outcome of this study. Retinal thinning is known to precede the formation of atrophy in Stargardt disease. These results with Remofuscin® are good news for patients hoping to finally have a treatment slowing the disease process.”

Dr Mario Fsadni, acting Chief Medical Officer for Katairo added, "Remofuscin® tablets appear to benefit both eyes, and the differences over placebo increase over time. These effects on the retina, with some achieving statistical significance despite the small sample size, have never been described before in this disease. It all adds credibility to our findings.”

“To date there is no approved treatment for Stargardt patients” said Prof Carel Hoyng from the Radboud University Medical Center and lead principal investigator, who presented the results at EURETINA congress in Amsterdam in the ‘Landmarks & Late Breakings’ session. “The results of the STARTT study give reason to hope that Remofuscin may be accepted for fast approval.”

The multinational study received funding from the European Union’s Horizon 2020 programme.

About STARTT

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

Remofuscin® in adults with Stargardt disease. Enrolled were 87 patients of which 61 completed the second year of treatment. Two thirds of patients received two tablets of Remofuscin® daily with each 10mg of the active ingredient Soraprazan. One third of participants received placebo.

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 toxic byproducts especially lipofuscin in the cells of the retinal pigment epithelium (RPE). 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), and had completed Phase I and Phase II clinical trials. Interestingly, soraprazan was also observed to result in removal lipofuscin of the RPE in non-human studies.

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 Rafaele, Milano (trial site), Dr. Maurizio Battaglia Parodi
- Leiden University Medical Center (trial site), Prof. Dr. Camiel Boon
- Southhampton University Hospital (trial site), Prof. Dr. Andrew Lotery
- Bonn University Eye Hospital (trial site), Dr. Philipp Herrmann
- Smerud Medical Research, Oslo (clinical research organization)
- Katairo (sponsor)

About Katairo

Katairo GmbH is a German 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 has also planned to develop Remofuscin for the treatment of dry age-related macular degeneration with the next study in phase 2. For more information, visit www.katairo.com.

Contact:

E-Mail: info@katairo.com Website: www.katairo.com