Information for dissemination to patients

Multi centre clinical study in Stargardt patients testing the safety and efficacy of Remofuscin® (soraprazan).

Remofuscin® is a drug currently being developed for the treatment of patients with Stargardt disease. A clinical study is being prepared/conducted in ophthalmic clinics in the Netherlands, Germany, UK and Italy (for contact information, see below).

The active ingredient of Remofuscin®, soraprazan, was being developed for gastro esophageal reflux disease in the past. In phase 1 and 2 clinical trials involving more than 700 normal or patient volunteers, the drug has been reported to be safe, with only non-serious adverse events being linked to the use of soraprazan.

The current study is the first study to test soraprazan in Stargardt patients. In a number of non-clinical studies, soraprazan was found to remove lipofuscin from retinal cells. High lipofuscin concentration in these cells is considered to be one of the main causes of damage to the retina in Stargardt Disease. In this study, both safety and potential benefit of the treatment will be checked. Not every patient joining the study will receive soraprazan: 1 out of every 3 subjects will receive placebo tablets which look like soraprazan tablets but do not have an active ingredient. Neither you nor your doctor will know what tablets you were given but, in case of any important side effects, this can easily be identified. This is necessary to determine if soraprazan can help patients with Stargardt Disease.

Participants in the study will have to take 2 tablets of soraprazan or placebo daily over the course of 52 weeks. Participants will be in the study for up to 14 months during which time they will be required to visit the study hospital at least 11 times. Overnight visits are not planned.

Eye exams and tests during the study include general eye exams, best corrected visual acuity, low luminance visual acuity, reading speed, quality of life questionnaires, microperimetry, quantitative autofluorescence, SD-OCT and Colour Fundus Photography. Some study hospitals may perform additional assessments; adaptive optics and pupillographic campimetry.

The study will be open for adults with genetically confirmed Stargardt Disease (at least two ABCA4 mutations) with a documented onset of disease before the age of 45 years. The study will accept both males and females. Further

inclusion criteria include a best corrected visual acuity of 0.2 to 0.8 (decimal) and an elevated value for autofluorescence, which is a biomarker for lipofuscin.

Patients will not be enrolled if they cannot tolerate drugs that reduce the acidity in the stomach. Current or a history of some medical conditions will also lead to exclusion from the study. These include abnormal blood results or some ocular conditions such as inflammatory ocular disease or high intra-ocular pressure. Breastfeeding or pregnant females will also be excluded from participation in the study. The patients eyes must be suitable for the tests to be performed in the study, they must dilate well in order to take clear pictures of the back of the eye. Other ocular conditions may prevent the participation in the study. Participation in other clinical trials will not be accepted.

Stargardt patients interested in participation in the study are referred to the following contacts at the following trial hospitals for more detailed information:

Ophthalmology Research Team
Southampton Centre for Biomedical Research
Mailpoint 218, C Level, West Wing
University Hospital Southampton NHS Foundation Trust
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This information letter has received ethical approval on 15/JAN/ 2019.