

# Designing a Clinical Trial to Evaluate the Safety and Efficacy of Oral Soraprazan in Stargardt Disease

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## Background

- Soraprazan has been reported in pre-clinical studies<sup>1-5</sup> to remove lipofuscin from RPE-cells, one of the hallmarks in the pathogenesis of Stargardt disease (STGD1)

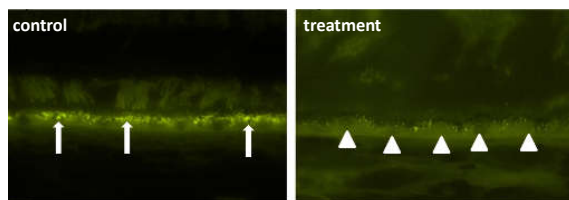
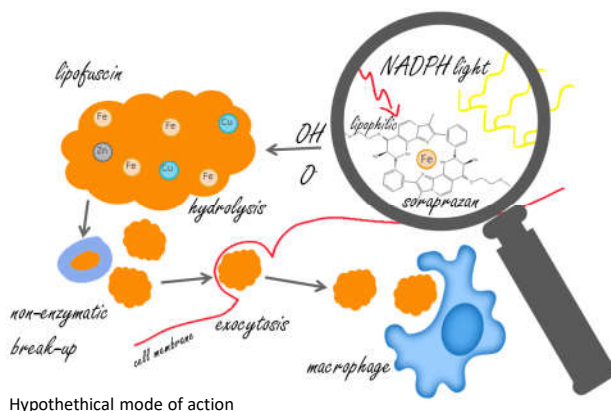


Image courtesy of U. Schraermeyer



Hypothetical mode of action

### Aim:

Start a phase II trial to evaluate the efficacy of soraprazan in reducing the amount of lipofuscin in RPE cells of subjects with STGD1

## Methods

- International consortium was formed
  - 5 investigator sites
  - Contract Research Organization
  - Start-up company & Sponsor
- A draft protocol and a business plan were fully costed and timelines defined. An application was filed with Horizon 2020.



## Results

- This project has received funding of €5.8m from the European Union's Horizon 2020 research and innovation programme under grant agreement No 779317
- Protocol for a randomized, proof of concept, double-masked, placebo-controlled, two arm, multicenter trial
  - 90 patients
  - 12 months
  - 20 milligrams, orally, once a day
  - Randomization 2:1

**Primary variable:** Change in qAF8 score from baseline to end of treatment (EoT) for soraprazan compared to placebo treated subjects.

## Conclusion

Soraprazan is potentially a new therapy for STGD1 which removes lipofuscin from RPE cells, thereby preventing or slowing disease progression.

A phase II trial will be conducted to evaluate the safety and efficacy of oral soraprazan in STGD1. Patient enrollment will be completed in 2019.

### References

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