

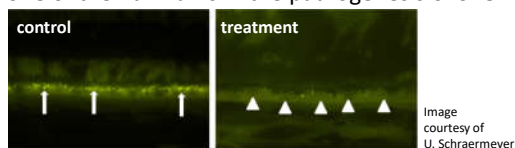
# Planning a Clinical Trial with Soraprazan for the Treatment of Stargardt Disease: Using a Patient-Specific Database

Patty Dhooge<sup>1,2</sup>, Carel Hoyng<sup>1</sup>, Clasiën Oomen<sup>1</sup>, Andrew Lotery<sup>3</sup>, Katarina Stingl<sup>4</sup>, Camiel Boon<sup>5</sup>, Maurizio Parodi<sup>6</sup>, Tobias Peters<sup>4</sup>, Wolfgang Klein<sup>7</sup>, Mario Fsadni<sup>7,8</sup>, Hans Müller<sup>9</sup>, Oliver Jungmann<sup>9</sup>

<sup>1</sup>Department of Ophthalmology, Radboudumc, Netherlands, <sup>2</sup>Donders Institute for Brain, Cognition and Behaviour, Netherlands, <sup>3</sup>University of Southampton, United Kingdom, <sup>4</sup>Center for Ophthalmology, University of Tübingen, Germany, <sup>5</sup>Academic Hospital Leiden, Netherlands, <sup>6</sup>Ospedale San Raffaele SRL, Italy, <sup>7</sup>Katairo GmbH, Germany, <sup>8</sup>International Pharm-Med Ltd, United Kingdom, <sup>9</sup>Smerud Medical Research, Germany

## Background

- Pre-clinical studies demonstrated the ability of soraprazan to remove lipofuscin from RPE-cells<sup>1</sup>, one of the hallmarks in the pathogenesis of STGD1



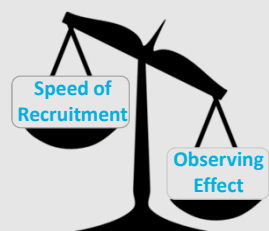
- Clinical trial with soraprazan for STGD1 is planned for 2019

### Inclusion criteria are difficult to establish

- Unpredictable effect of criteria
- STGD1 is a rare disease

### Aim:

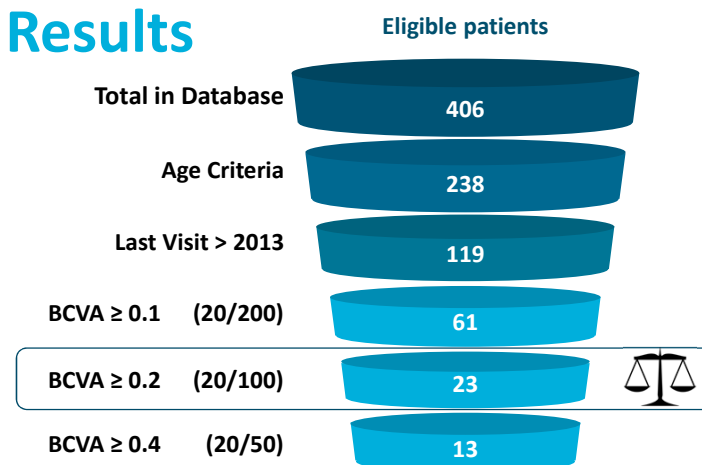
Fine tune inclusion criteria for a clinical trial to evaluate the safety and efficacy of soraprazan in the treatment of STGD1



## Methods

- A unique STGD1 database has been created in Nijmegen with clinical data of over 400 patients with STGD1
- Proposed inclusion criteria for Soraprazan study:
  - age  $\geq 18$  years
  - onset symptoms  $< 45$  years
  - BCVA 0.8-0.4 (20/125-20/50)
 } cross checked to STGD1 database entries
- 90 subjects over 5 centers required for statistical significance

## Results



Inclusion criteria were adjusted to BCVA  $\geq 0.2$  to balance the speed of recruitment rate with the chances of observing a treatment effect

## Conclusion

Academic centers with an interest in conducting clinical trial research are strongly encouraged to set up patient databases in the disease of interest

### Final inclusion criteria for Soraprazan study

- BCVA  $\geq 0.2$  (decimal)  $\geq 20/100$  (Snellen)
- Age  $\geq 18$  years
- Age at onset  $< 45$  years
- Genetically confirmed clinical diagnosis of STGD1

### Reference:

1. Julien, S., and Schraermeyer, U. (2012). Lipofuscin can be eliminated from the retinal pigment epithelium of monkeys. Neurobiol. Aging 33, 2390–2397.

### Contact:

Patty Dhooge, MD  
 Patty.Dhooge@radboudumc.nl  
 +31 (0)24 36 10241  
 Radboud university medical center

