

DRUG4SIGHT: Light-regulated drugs to restore sight

Challenge

In retinal degenerative diseases like Retinitis Pigmentosa, photoreceptor cells are progressively lost, leading to visual impairment with very limited treatment options. However, the inner retinal neural circuitry persists, and the remaining neurons can be sensitised using drugs that target neuronal proteins.

Market

Retinitis Pigmentosa is a genetic rare disease, and it is estimated to affect around **1.5 M people** worldwide, where symptoms are usually present from childhood. It is forecasted that the **sales** of Retinitis Pigmentosa products will **reach up to \$2.3B** by 2030.

There is a lack of new pipeline products and current options including gene therapies are very expensive, invasive, and offer low efficacy and photosensitivity.

Asset

As the inner retinal neural circuitry is not degenerated, **neurons can be activated using small-molecules**, that target specific neuronal proteins.

Dr. Pau Gorostiza discovered together with his team a series of **light-responsive small molecules** have been developed to **restore sight**, allowing functional restoration of visually guided behavior. This is achieved by mimicking the rod and cone function with the light activable compounds.

Our topical administration could disturb the market with **reduced costs, user-friendly application, and shorter regulatory pathway**.

Asset Value

- ❖ Achieve **functional restoration** of visually guided behavior in small animals
- ❖ Administered **topically**
- ❖ Highly **selective target**, strategically positioned "upstream" in the retinal circuit signaling
- ❖ Retinitis Pigmentosa is a **rare disease** and will allow for simpler and faster safety assessment at the European Medicine Agency than usually encountered with systemic drugs.
- ❖ This product is being developed with the collaboration of FARPE and ONCE, the two biggest patient associations in SPAIN



Normal vision



Age-related macular degeneration



Retinitis pigmentosa

Uses

- ❖ Retinitis pigmentosa
- ❖ Age-related macular degeneration
- ❖ Sight improvement in healthy population

Team

Pau Gorostiza - Scientific Leader

Asli Raman - Tech Transfer Manager

Eduardo Salas - Head of Tech Transfer

Stage of Development

In vivo efficacy studies with zebrafish and mice completed with positive results
Preclinical studies ongoing

Regulatory Path

Rx

Intellectual Property Status

IP application on-going

Exploitation Plan

Licensing

Open to technical co-development or investment for preclinical phase completion

Contact

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