

# 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.

DRUG4SIGHT project has developed light-regulated drugs targeting receptors expressed in retinal bipolar neurons, which offer unparalleled response selectivity and upstream signalling control of the retinal circuit.

### Market

Retinitis Pigmentosa is a genetic rare disease, and it is estimated that disease affects 1.5 million people worldwide where symptoms are usually present from childhood. 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

Dr. Pau Gorostiza discovered together with his team photo-switchable one-component small molecules to restore sight. Our topical administration could disturb the market with **reduced costs**, **user-friendly application**, and **shorter regulatory pathway**.

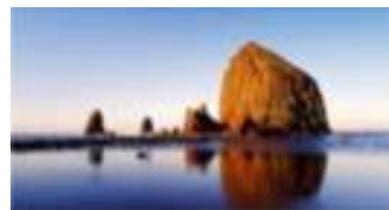
### Asset Value

Our compounds are the only ones allowing functional restoration of visually guided behaviour by topical application in the cornea. This feature together with the status of Retinitis Pigmentosa as rare disease will allow simpler and faster safety assessment at the European Medicine Agency than usually encountered with systemic drugs. In addition, our pharmacological target is only expressed in the mammalian retina and in contrast to ion channels and receptors used so far, it is strategically positioned "upstream" in the retinal circuit signalling. This fact anticipates **high pharmacological selectivity**, **safety profile**, and **optimal visual acuity** that takes advantage of the computation properties of the retinal circuits.

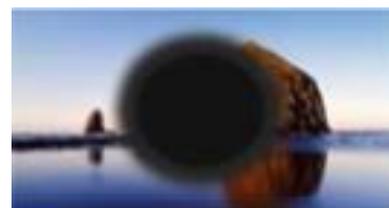
The DRUG4SIGHT project aims to discover and characterise a series of compounds that can stimulate proteins still present in the degenerated retina and make the non-degenerated remnant neurons act as photoreceptors, light-sensitive cells.

Institute for Bioengineering of Catalonia (IBEC) is a research centre of excellence set up to conduct interdisciplinary research at the cutting edge of knowledge in the bioengineering field. Dr. Pau Gorostiza, group leader at IBEC, has a longstanding experience in photopharmacology for basic research purposes and as a treatment for different pathologies or conditions. Thanks to the partnership with Barcelona Macula Foundation, his team is in regular contact with ophthalmology experts to discuss the development of this project.

**Collaborating Patient Associations:** FARPE and ONCE



Normal vision



Age-related macular degeneration (elderly)



Retinitis pigmentosa (children, young adults)

### Uses

Retinitis Pigmentosa  
Sight improvement in healthy population

### Scientific Project Leader

Dr. Pau Gorostiza

### Stage of Development

*In vivo* efficacy studies with zebrafish and mice completed with positive results

### Regulatory Path

Rx

### Intellectual Property Status

IP application on-going

### Exploitation Plan

Licensing

### Contact

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