# Therapy for retinal degeneration in ciliopathies

**Pharmacological therapy to slow significantly retinal degeneration linked to protein trafficking defect inducing a Unfolded Protein Response**

## KEYWORDS

*Photoreceptor cell, Retinitis pigmentosa, Cellular protein overload, Endoplasmic reticulum stress, Apoptosis, rare diseases*

## PATENTS

**WO2013124484**

## INVENTORS

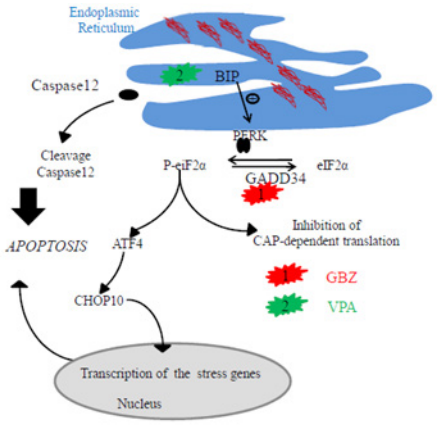

**H. Dollfus**
**V. Marion**
**A. Mockel**

Laboratory
of Medical Genetics

Unistra / INSERM


## TECHNOLOGY

Impaired intraciliary transport observed in Bbs12-/- murine model
- results in protein retention in the endoplasmic reticulum
- this leads to a proapoptotic unfolded protein response (UPR) triggering death of the photoreceptors
- BiP, and PERK-mediated phosphorylation of eIF2a have been identfied as 2 key UPR actors (Mockel et al., 2012)
- GV-ReT is a combination of clinically validated molecules (Valproic Acid and Guanabenz) for the treatment of cilia-related retinal degeneration




## APPLICATION

- Pharmacological therapy for ciliopathy related retinal degeneration

## INNOVATION ADVANTAGES

- A combination of two approved drugs, with a well defined MoA
- Effective in protecting retinal cells from dying and significantly increasing light detection capacity
- Aims at preserving the vision loss and slowing the retinal defects to cure secondarily with a more targeted therapy
- Positions on early onset of the retinal degeneration
- Applicable to different paradigms of retinal degeneration : UPR-inducing ciliopathies, retinal degeneration linked to protein trafficking defect inducing a UPR

## DEVELOPMENT STATUS

- Compelling ex vivo and in vivo data establishing the efficacy of this drug combination
- Efficacy assessed on different cilia-related retinal degeneration (Bardet Biedl syndrome, Leber Congenital Amaurosis )

*Partnership : program available for out-licensing*

OT_Dollfus_20150310


Parc d'Innovation
650 Bd Gonthier d'Andernach
67400 ILLKIRCH - FRANCE
**www.satt.conectus.fr**

**CONTACT**
Vincent Bischoff
Business Developer
(+ 33) 3 68 41 12 66 - vincent.bischoff@satt.conectus.fr