For patients

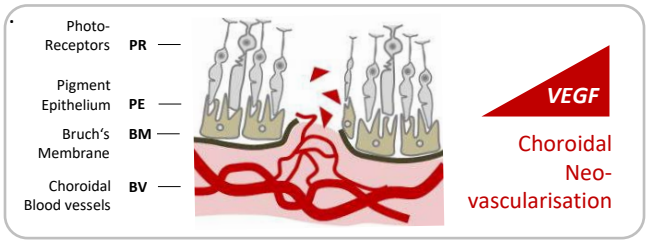
# Project Target AMD

Transposon-Based, Targeted
*Ex Vivo* Gene Therapy
To Treat
Age-Related Macular Degeneration

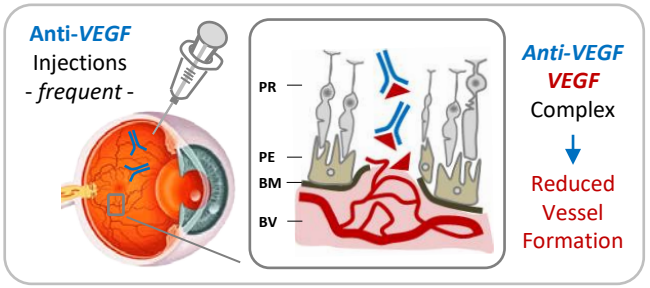
**Piloting the 1st EU Study in Human**
Clinical Trial Phase Ib/ IIa

## Purpose

Patients with wet age-related macular degeneration (AMD) suffer from the abnormal growth of blood vessels (neovascularisation) resulting from an increased level of the *vascular endothelial growth factor* (*VEGF*) ("pro-angiogenic" factor).




Current treatments comprise frequent injections into the eye of anti-*VEGF* drugs to inhibit the neovascularisation and prevent the subsequent retinal damage.

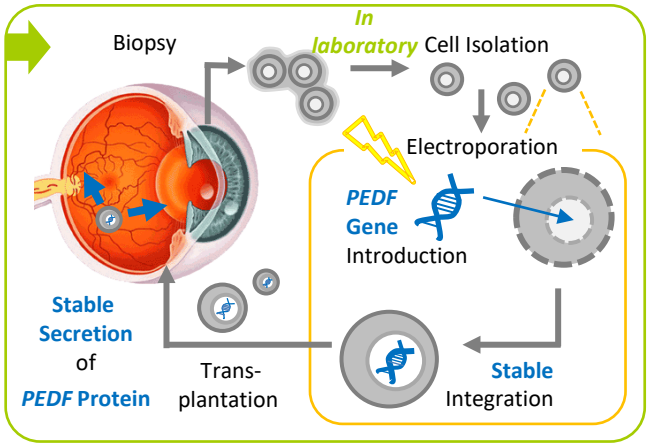



The aim of TargetAMD was to develop a non-viral gene therapy to treat wet AMD, by transplanting the patient's own (i.e., autologous) genetically modified retinal pigment epithelial (RPE) cells (i.e., cells that help to preserve a functional retina), to express and secrete continuously the *pigment epithelium-derived factor* (*PEDF*) that is preventing the function of VEGF and thus, stopping the growth of newly formed blood vessels.

## TargetAMD Approach

TargetAMD aims to accomplish the 1st European pilot study in human to evaluate the safety of the technique to modify the RPE cells, the non-viral *Sleeping Beauty* (*SB100X*) transposon system in a gene therapy.

- RPE cells will be isolated from biopsies from advanced-stage wet AMD patients. The cells will be genetically modified in a designated laboratory and immediately transplanted back to the patient underneath the retina within the same surgery.




- The human gene that codes for the PEDF protein will be introduced into the genome of cells isolated from the patient by equipment (Cliniporator®) and reagents developed by the TargetAMD partners.
- The safety of the approach was enhanced by combining the *Sleeping Beauty* (*SB100X*) transposon system with the pFAR4 system, gene sequences free of antibiotic resistance genes (the "vector").
- Laboratory studies were performed to proof safety of the vector.
- Novel plasmids and reagents are produced under highly controlled, i.e., GMP (Good Manufacturing Practice) conditions.



*EU 7th Framework Programme*

## Benefits

The TargetAMD consortium enhanced the safety of transposon-based gene delivery. Newly established safety standards will facilitate translation of basic research into future clinical applications.

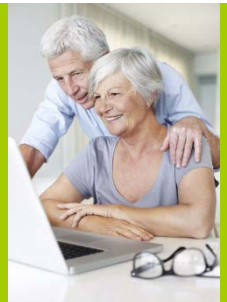
TargetAMD essential features:

| | | | |
|---|---|---|---|
| Combination of the pFAR4 & *SB100X* technology | Optimisation of transfection (genetic modification) parameters | GMP production of plasmids and reagents | Applying for approval of clinical trial based on safety studies |

TargetAMD advantages:

- Establishment of a new, safe and practical approach to gene and cell therapy.
- Development of standardized procedures.
- Will be the first approval for this new clinical treatment in Europe.
- Only one surgical session will result in a long lasting and cost-efficient treatment.
- Improvement of the patient's quality of life allowing active ageing.
- Market launch of novel, optimised, approved devices and reagents.
- Provide information for the benefit of future patients and clinical trials.
- Transfer of protocols developed for TargetAMD to other clinical disciplines.

**Cost-Efficient Non-Viral Gene Therapy To Restore Vision In Patients With Wet AMD**



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## Consortium

The TargetAMD consortium is an interdisciplinary team of 13 partners, companies, scientists and clinicians, supported by project management, from 8 European countries. TargetAMD aims to transfer a cell-based gene therapy for patients suffering from wet AMD into a phase Ib/ IIa clinical trial.

### Project Partners

- University of Geneva, CH
- RWTH Aachen University, DE
- Max Delbrück Center for Molecular Medicine in the Helmholtz Association, DE
- Paul Ehrlich Institut, DE
- Universidad de Navarra, ES
- IGEA S.p.A., IT
- UD-Genomed Medical Genomics Technologies Ltd., HU
- Centre National de la Recherche Scientifique, FR
- 3P Biopharmaceuticals, S.L., ES
- Genosafe SAS, FR
- Rudolf Foundation Hospital, AT
- Stichting Amsterdam Biotherapeutics Unit, NL
- University Hospital RWTH Aachen, DE

www.targetamd.eu

## Project

TargetAMD - Transposon-based, targeted *ex vivo* gene therapy to treat age-related macular degeneration (AMD).

### Duration
Start foresenn in fall 2023

### Total Cost
7.73 Mio. Euro

### EC Contribution
5.97 Mio. Euro

### Call
FP7 Health-2012-Innovation

### Coordination
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