

Publishable summary

RdCVF is the abbreviation for a protein called Rod-derived Cone Viability Factor and at the same time the name of an European research network, which aimed at developing a therapeutic strategy for preserving the function of cone photoreceptors in patients suffering of retinal degenerations like retinitis pigmentosa (RP). This research network is coordinated by the Institut National de la Santé et de la Recherche Médicale (Inserm) through Prof. José Alain Sahel (Director of the Vision Institute, Paris, France). The consortium consists of 5 participants from 3 different European countries: France, Germany and Portugal

Retinitis Pigmentosa (RP) is the most common form of inherited retinal diseases that affects altogether 1,500,000 people worldwide. It is a genetically heterogeneous group of disorders having common characteristics that lead to irreversible loss of vision. Initially, in affected people, rod photoreceptors that are responsible for night vision degenerate, followed by cone cells, responsible for central and light-adapted vision. RP is still an untreatable disorder and preventing cone cell death is a very promising therapeutic approach, since even when 95% of the cones have been lost in patients the vision remains substantial.

The RdCVF consortium has faced a major technical issue, namely that this protein is hydrophobic and insoluble. This, as far, unknown nature of the functional RdCVF protein made impossible, using the tested methods, its production in a quantity necessary for a therapy. Through this unexpected result, the consortium wanted to refocus its work plan proposing 2 alternatives:

- 1) Design and synthesis of molecular agonists of the protein
- 2) A gene-based, instead of a protein therapy.

It was estimated by EU-appointed experts that these alternative strategies could not bear fruit during the span of time of the funded project.